DEPARTMENT OF HEALTH AND HUMAN SERVICES

ADVISORY COMMITTEE ON BLOOD SAFETY AND AVAILABILITY

Twenty-First Meeting

Volume II

8:32 a.m.

Friday, August 22, 2003

Hyatt Regency Capitol Hill Hotel
Capitol Room
400 New Jersey Avenue
Washington, D.C. 20001

P A R T I C I P A N T S

Mark Brecher, M.D., Chairman

Larry Allen
Judy Angelbeck
Celso Bianco, M.D.
Ronald Gilcher, M.D.
Edward D. Gomperts, M.D.
Paul F. Haas, Ph.D.
William A. Heaton, M.D.
Christopher Healey, J.D.
W. Keith Hoots, M.D.
Jeanne Linden, M.D.
Karen Shoos Lipton, J.D.
Lola Lopes, Ph.D.
Gargi Pahuja
John Penner, M.D.
S. Gerald Sandler, M.D.
Mark Skinner, J.D.

Non-voting Government Representatives

James S. Bowman III, M.D.
Jay Epstein M.D.
Mathew Kuhnert, M.D.
Harvey Klein, M.D.
Captain Lawrence McMurtry
LTC Ruth Sylvester

C O N T E N T S

P R O C E E D I N G S

DR. BRECHER: Here's another public service announcement that's related.

[Videotape played.]

DR. BRECHER: Okay. It's a good thing a little dog didn't get it before the big dog.

With that little diversion, we're going to move on to looking at new technology potentials, and the first speaker is Jeff Miripol from Terumo, if he's here.

Jeff is not here. Why don't we--who's the next one? Why don't we get Jerry Holmberg? Is he here?

Oh, this is good. Anyone got any other film clips?

[Pause.]

DR. BRECHER: So much for the best laid plans. Well, we could go ahead and have some public comment, if there are any--nobody here for that either, huh?

Anyone got any good jokes? This is a joke.

I apologize. It's looks like we're going to just go on hold until 9 o'clock. So much for the best laid plans. Is there anyone for public discussion? Anyone want to make a public comment?

[No response.]

DR. BRECHER: We tried. Does the Committee want to discuss what we've heard so far? We could start on that. Presumably we're going to hear about some alternative technologies, updates from Haemonetics on the frozen deglycerolized red cells.

MS. LIPTON: I was just going to suggest that in light of everything we've heard to date, I didn't see any need for a resolution on the part of this Committee with respect to the reserves. But I offered to take all of the comments that we heard yesterday back to the disaster task force and ask them to consider and then to come to this Committee perhaps with a recommendation or a series of recommendations for consideration, if that would be helpful.

DR. BRECHER: Further comments? Jeanne?

DR. LINDEN: In the discussions yesterday, I was giving some thought to our most recent modest disaster, you know, the great power outage in the Northeast, and thinking about what happened there and the possible ramifications had that been more extended, because the transportation options were extraordinarily limited, with no planes, no trains, and gridlock traffic-wise with a lot of the bridges closed. And since most of the businesses, in fact, really all of the businesses closed, there couldn't be new collections, and we lost several thousand potential units that could have been collected and weren't. So that was another example of what--you know, the blood we were using is the blood on the shelf. And it would have been possible to get more blood in over time. But had that been extended, that was really an issue. And I think it was a good learning experience to look at, well, when we lost the infrastructure, you know, what can we do in those situations?

I think examples like that and certainly some of the ones that Ron has shared are really

good learning experiences. We can look at the actual issues that came up.

Something else that came up with the loss of power is the loss of computer services. We even in Albany lost access to the Internet. In New York City, they had nothing, no phones, although AOL was working so people could use their home e-mail. But because the laboratory testing is all done in the state, we have no laboratories doing infectious disease testing for blood donations in New York State, we couldn't get the test results back. And in small numbers for certain emergency donations, you could get them by phone or fax in some cases, if you had phone or fax. Those of you who read my article about faxing, you know, know that faxing is fraught with a lot of problems. And, obviously, when we're talking about thousands of units, that can't be done. It would be very error-prone if you were to do manual transcriptions and basically a lot of units that were even on the shelf couldn't be used because the test results couldn't be accessed.

So there's all sorts of ramifications as we rely on electronic technology that, when you lose the power, you all of a sudden lose that electronic technology. So it's just something I was thinking about over the last day or so that, you know, I think is a good learning experience.

DR. BRECHER: Karen?

MS. LIPTON: Well, in fact, because the disaster task force convened immediately, we were aware of these issues, and we're developing some modules, particularly, as I said, on the water and the computer situation.

I think it was Ron who said this, that, you know, planning for a disaster isn't the plan. It's knowing--it's really knowing how you react and who you get to. And so I don't think that we can understand every situation. I think the most important thing we now do is at least we have a communications hierarchy that allows us to communicate even if all those systems go down. And we just have to know that we're in touch.

But we are aware of that, and we're

actually doing a debriefing on this. Every time something happens, we do a debriefing on it, and we will go out to the regions as part of the process to ask them what their experiences were that they could share with other blood centers.

DR. BRECHER: Ron?

DR. GILCHER: What I wanted to say was that, in fact, that's absolutely correct. The disaster never fits the plan, and we've learned that from the two major disasters we've had in Oklahoma. But, in fact, the process of planning is so important because you really then have to--you have things in place so you can make the plans as the disaster is in progress. It's critical that all the staff know exactly where to go, what to do; when something happens everybody has a place to go, and then having redundant systems in place. There's just no generator, fuel supplies and so forth. We have done that at our blood center so we can operate literally for a couple of weeks without any additional power. But that's a big problem to us because we do take power outages in Oklahoma

quite frequently during the summer months.

MS. LIPTON: We just figured we're just going to establish New York Blood Center as our test case for everything because it always seems to happen to them.

DR. BRECHER: It makes you wonder if in their disaster plan--I imagine there was huge gridlock in Manhattan--if they include bicycle messengers to deliver the units of blood around the city.

We'll take a 30-second pause while they fix the lines.

[Pause.]

DR. BRECHER: Jerry, you're on.

DR. SANDLER: Yesterday we didn't have a chance to ask a lot of questions to Dr. Williams, and I was wondering if I could pick up on that.

Prior to the presentation, many people correctly identified the fact that we don't have a nationwide information system that tells us how much blood we have and where.

On the other hand, we really have three.

For years, Marian Sullivan's operation has been functioning. The Office of the Secretary has sponsored the sentinel sites, and Dr. Goldfinger and myself and other blood bank directors have been participating and sending our information, and there's been publications, and now FDA has taken an initiative. And I was wondering if you could perhaps define if there's different functions for each of these national data collecting systems that aren't.

MR. WILLIAMS: I don't have the advantage of having the slides here, but in one of the early slides, what I did was set out what I thought would -- [sound out] -- of a national system. And, yes, we have several systems in place. Each one of those measures a certain aspect of the blood supply and is useful for a specific purpose but doesn't cover the whole waterfront of what we might need.

For instance, the NBDRC program, as I know it, speaking to the monthly data collection, is from 26 sentinel sites. It's rather comprehensive data on collections and supply and variations over

time.

The one aspect of that is that the data is available sometime after the reports are actually issued, so it's not realtime. And, Karen, if you want to correct me on any of these, I'm going from memory here. But one disadvantage is that it's not realtime information. It's a good sample, but it is a sample. It's not a national population-based assessment that will really give assessments at the local and regional level.

The HHS program also is a sentinel program at 29 sites. It measures hospital inventory for all blood types in inventory by a standardized definition. This is published on a daily basis. Again, I think one potential shortcoming of that design is that it, again, doesn't represent the local and regional shortages that might occur. While it does give a national picture of, you know, when things go up, when things go down, if you have a crisis in a specific area you really still don't know what's going on. And Mac is closest to that and might wish to comment as well.

The program that was described in some depth at an earlier meeting--and I just went over it very quickly yesterday--the TRANS-Net program is specifically designed at the current time to measure shortages at primarily the transfusion service level, although it would also function at the blood center level. It's designed to be a population-based voluntary program so that--let's assume for the moment we had 100 percent participation, which is unlikely. We would be able to capture individual local and regional shortage issues, have that available in both the database and a geospatial map so that we would be able to pinpoint when trends were beginning to develop, compare them with a historical record, and be able to do some predictive modeling, and really have an in-depth perspective of how the nationwide picture looks. If there's a shortage in one area, there may be a worse shortage in another area. And I think one big advantage of the TRANS-Net program is it gives an assessment of what impact those shortages are having rather than just saying, okay,

there's a shortage in this area.

If it starts to approach a point where patient care is actually being compromised, you know that through this program, and I think that's one advantage.

None of these are ideal systems which capture all of the elements, and I think, you know, the plea that was being made is to build a comprehensive system that as efficiently as possible through a combination of these programs will capture the elements that we need, both to assess what's going on in a routine situation as well as in extreme crisis, just so it can be managed efficiently.

DR. BRECHER: Mat?

DR. KUHNERT: Mat Kuhnert from CDC. I just wondering in sort of thinking about the discussions yesterday about what analogies there are and sort of comparable systems in other fields. You know, there was some mention about Wal-Mart and business models, which I don't know if we can hope to model those efficiencies, but also to other

systems in health care like pharmaceuticals, vaccines, but also other biological products like organs. There's an organ transplant network and sort of--it's a different, I think, scale. This is a far greater scale. But I wonder if that could be looked at and compared to as far as whether there could be any analogies to blood from the experience of organs and also tissues.

The other thing I wanted to mention, it seems like there's a lot of existing efforts for reserves, and, you know, maybe there isn't one answer. There's no one hemovigilant system in the United States, and maybe it is going to end up being that there's going to be different approaches. But the key point will be connecting those together, and the key point will be whether the systems can talk to each other. So I think data exchange is going to be the biggest issue, I think, to tackle.

DR. BRECHER: Mark?

MR. SKINNER: I just want to make a comment, and it's not really the focus of this

discussion, but we did have a presentation by Julie yesterday afternoon on PPTA, on kind of the related issues. It wasn't the focus of all the discussions, but I hope the Committee is mindful that the issues--there are a lot of parallels between the discussions. And while the nature of the shortage may be different because of the supply in the system for the plasma-based therapies, we have experience with facing sustained shortages, like the two-year shortage of recombinant products. And there really aren't the same kinds of contingency and back-up plans there, as well as the same issues of getting product to the rural hospitals and other places when you need recombinant products. That whole infrastructure and that side of the equation in managing a sustained shortage on the plasma products as well as the emergency need in areas that don't have ready access for certainly the coagulation therapies, that piece doesn't exist as well. So not that this is where we're going to answer that question, but I just wanted to kind of point out

what was behind the rest of what Julie said yesterday from my impression.

DR. BRECHER: Okay. I think we're ready to move on to our morning speakers on the new technology potentials. Jeff, are you ready to go?

MR. MIRIPOL: Yes. It looks like we actually have some technology that might work. of course, I think like a lot of you, I had the So Big virus as well. I'll just give this a moment. It's thinking. It's going through the...

DR. BRECHER: While it thinks, if anybody would like that little public service announcement, let me know. I can e-mail it to you.

MR. MIRIPOL: Good. All right. Again, thank you very much for the opportunity to speak to you. When Mac McMurtry called me and asked me to speak, I sent him a little outline as to what I was going to talk about, and he said that's fine. So I hope this is fine.

I wanted to give you an overview, at least from a manufacturer's perspective, of issues as we see issues in terms of I guess what one would

broadly call the availability of products for the blood supply, to ensure the blood supply.

Just as a little overview, you know, blood is a unique product. It's obviously the basic raw material that comes from humans. It's a complex drug, non-homogeneous product, blood component therapies obviously required for a broad range of medical conditions. But it is a unique product in that it's treated as a drug, but it's not a homogeneous drug.

Blood products have a very finite shelf life, and this is the big issue in terms of ensuring supply. Platelets have a five-day storage period at room ratepayer; red cells, 42 days at 4 degrees; plasma, obviously, frozen, fractionated, et cetera. And then the availability itself is an uncertain, very unclear situation often, very tenuous, as obviously the reason for this conference.

Collecting blood, obviously, requires that the donor is available, the donor is willing, and the donor hasn't been frightened off. I think one

of the problems we're seeing in this country is that the donor is often in short supply for a variety of reasons. And I think a lot of times it's because of fears within the donor community.

There's a unique interaction between the donor and the blood center. Obviously, the blood center tries to appeal and does appeal to the donor's sense of community, helping others, et cetera, and the first dictum, which certainly applies to medicine and applies here, is, you know, you don't want to do any harm to the donor and you don't want to drive the donor away.

The products required for blood collection are various, and there's a large number of them. This just lists a few things that are used in blood collection: obviously, the collection bags, the materials you need to do the skin preps, the phlebotomy equipment, processing equipment, apheresis equipment, testing equipment, test kits, labels, computer systems, refrigerators, freezers, et cetera. That just scratches the surface. But essentially to collect, store, inventory, ship,

transfuse blood, it's a very, very elaborate process. In some ways, it's obviously much more complex than just making a vaccine or making a specific drug because of the need for the raw material and how you have to process it, et cetera.

Many elements of the supply, in terms of the suppliers, the materials, the supply chain, are really concentrated in a few companies. This holds for both the blood bag suppliers, holds also for the testing companies, the apheresis companies, et cetera.

This is clearly a very specialized industry, and it has very clear specialized manufacturing expertise. The systems required are specialized to collect blood, and, of course, all of this must meet stringent regulatory requirements and oversight. I'm not telling anything new to you folks, but I thought it was helpful to at least kind of summarize some of the issues that face all of us, all of you, in the area of blood availability.

The entry barrier for new suppliers is

relatively high. This is a business where the margins aren't that great from a supplier standpoint. If you were to get into a business, this is not a business I recommend you get into.

There certainly are some companies that are looking at the U.S. marketplace. Certainly MacoPharma in France is. There's other companies that are looking at the marketplace. But I think if, you know, one is rationally focused in a business model, this is not a business you'd get into.

There's a lot of regulatory oversight, and the regulatory requirements do provide a very important area of consistency, and they do limit variability. But this is not a free-for-all industry, and the supplier-user relationships are extremely close, whether it's the apheresis manufacturers, the blood bag manufacturers, the manufacturers of the test kits, et cetera. The relationships with us and the blood center, blood center organizations, is extremely close.

I want to focus now in the areas that

Terumo specifically deals in, and that's blood bags. There are right now in the U.S. three FDA-approved suppliers: Baxter, Pall, and ourselves. So I guess one could say that you've got at least an assured supply of product because of the variety and variability of suppliers.

Terumo's capacity to supply is probably in the neighborhood of 5 million bags to the U.S. We supply about 3, 3.1 million right now. Baxter clearly has a larger supply capability for the U.S., and I'm not sure what Pall's capacity is.

The blood bag suppliers, our blood bags are drug products. They're unique products in many respects. The manufacturing facilities to make blood bags are quite unique and expensive manufacturing operations to build and to maintain. Essentially we're making IV solutions of small volumes under very stringent FDA control and requirements.

Many supply elements--and it's not just for Terumo because our blood bags are made in Japan, but many supply elements even with our

competitors are made outside of the continental U.S. This is something you have to consider because the whole supply chain is, in my view, one of the key issues in terms of supply of blood and blood products. The requirement in the U.S. is for over 15 million blood bags a year, so we're not talking about an insubstantial number of products.

The raw materials that are required, the plastics, the solutions, et cetera, also must meet very specific requirements. And the suppliers of the raw materials, at least certainly of many of the raw materials, are also specialized, and their raw materials have to meet certain requirements that are much more stringent than, say, the plastics use to make beach balls or things like that.

So, as I indicated, you have to look at the total supply chain. It's a very, very crucial and critical element.

Also, the quality systems that we and our competitors have are very complex and they're very detailed and have very extensive oversight from the

FDA and other regulatory agencies.

So what I'm getting at here is that from the manufacturing standpoint, I think that the public has a high level of assurance that, say, the blood bags or apheresis kits, if you will, or whatever, have a very high level of quality and assurance. And one I guess might be asking: Are we concerned about sabotage? Are we concerned about some other issues?

The manufacturers all have plans for various emergency situations, and I understood that that was one of the things that, you know, Mac wanted me to sort of address. However, what happens when transit is completely shut down? September 11th, no flights were flying, no trucks were running, et cetera. We actually were able to supply blood bags, even though our bags come from Japan, because we had large inventories in the U.S. You know, we had folks getting in vans and bringing blood bags to blood centers, et cetera.

But this is a critical element in terms of planning and supply. We need to have, I think, a

better plan to ensure that, you know, products are made available.

Now, that's just the raw material. The blood itself then, getting the blood from the blood centers to hospitals, that was probably also affected to some degree.

Also, what happens when you have rumors of contamination? And what happens when donors become fearful of donating? Again, just recently, in January and February, this issue of white particulate--you know, blood is non-homogeneous substance. This was not an issue of contamination. But, again, it's an issue of, I think, direction and understanding the whole supply chain and also leadership.

So what are the present issues in supplies--or problems in terms of supply assurance, at least from our view? One of the major issues I am obviously speaking about here is getting the blood bags and supplies from outside of the U.S. to the U.S. It takes twice as long now as it used to prior to 9/11 for us to get blood bags, for

instance, into the U.S. I think some of our competitors and other suppliers are also seeing situations much like this.

We believe we have to simplify, develop a list of ways to release incoming products so that the customs officials and the FDA, et cetera, can work together to ensure a more assured supply and a more simplified supply chain. Again, it's important to ensure safety, but if the product is not available, then it's obviously counterproductive.

Also, we believe--I guess to that point, we believe we need a better coordination of the regulatory and customs processes and procedures. Certainly this conference has been discussing yesterday and I'm sure more today the issue of donor availability. This is not our area of expertise, but certainly one would think that we need better national programs for donor awareness and recruitment. We also think that we need better media integration of the blood resource needs, and we also need to reduce hysteria. I think every

time there is a new pathogen--you know, West Nile, et cetera--from every indication I have from our colleagues in the blood centers, this leads to a reduction in donors. And, again, I think this has to do with perhaps multiple messages or a lack of a consistent message.

As you've been hearing here, blood is available but it's not available in the right places. So the systems are complex, the response times are reduced, and, frankly, there's no national coordination of blood policy. To that point, in terms of a national blood resources disaster plan, from our perspective there really is no plan. There's a lot of efforts, and certainly this is a very good attempt to try to bring this together. But we don't really have a unified process or unified group that gets together and really pulls together the requirements chain, the blood chain, and the supply chain.

So what is the real concern? The concern is bioterrorism. Well, what does the bioterrorism concern really mean? Does it mean the blood bags

or the test/assay materials, et cetera? Are they being sabotaged? I don't think so, and they're very closely controlled during manufacture. I think that's the least of anybody's worries.

You know, is the fear of sabotage of blood products once the products have been made in the blood center or sent to the hospital? Well, that's a complex and, I think, a very, very difficult question. And I'll make a few points subsequent to this on that, but I think it's going to be very, very difficult to try to develop systems to ensure that every unit of blood has not been in some way sabotaged by an unhappy worker or something like that.

So, again, as I pointed out earlier, as you all know, blood is a complex product, and it is not homogeneous. So if you see white cells in it, don't get upset.

In terms of our suggestions and our thoughts on this area, certainly we believe a national blood resource plan would be important and, frankly, mandatory as things become more

complex and fearful in our world. However, we believe that this ought to be a very simple plan in its basics.

Remember that you may not have power. Look at just what happened last week in New York. We have colleagues here, of course, who are going to talk about certainly freezing systems and other kinds of systems, even apheresis systems, et cetera. Blood bag collection is low-tech and reliable. You don't need a lot of equipment. That's a big advantage. It's easy to do in lots of different places. You don't have to have complex environments to collect blood in blood bags.

We believe there should be a supply depot situation in the U.S. based--sort of similar to what the military did some years back, where this is coordinated amongst all the suppliers and the blood centers. This could be done at blood centers, probably most appropriately done at blood centers.

We think that a centralized national group with clear leadership to coordinate blood

availability and these plans should be put together, should obviously include the military, blood centers, suppliers, the providers, the regulatory agencies, et cetera. But--and this is something, I think, that became very clear 9/11--I think we need to have a single focal point or a single voice to give clear information in these situations. And I think that's a very, very key issue in terms of the donor assurance and making sure that the donors are coming in and are willing to donate under these kinds of circumstances.

In terms of new technologies, we believe that the major issue is actually patient safety, and certainly one of the biggest problems is the mistransfusion of blood products. So we believe that resources ought to be put at the transfusion area in terms of assuring the safety of the blood products there, and really it has to do with the assurance that someone who is an A patient gets an A unit of blood and not a B unit of blood and so forth.

New technologies in terms of bacteria

testing, admirable. We ourselves are not working directly in this area. We believe it's important. Virus testing procedures and making sure that viruses or bacteria or other pathogens are inactivated, useful, expensive, and, again, does not answer the issue of sabotage of blood products.

Our thinking is that one of the areas, although we ourselves are not working directly in this area, that should be looked at and perhaps funded more strongly is the area of bio-sensors. We think that there are some technologies out there that are very, very promising that could be applied to blood products, both to identify pathogens and perhaps other kinds of drugs, et cetera.

Our efforts in terms of technologies are basically fairly straightforward and relatively simple. In the blood bag collection systems, we've developed a diversion blood bag system, as our other colleagues have. The intent here, of course, is to remove the initial bolus of blood from the donor, that bolus perhaps being one that has more bacteria, et cetera, associated with it. A simple

system that we believe does improve safety.

We obviously also are working on and have approval for some blood filter systems, for white cell removal. This we believe increases some marginal safety from the standpoint of transfusion reactions, et cetera, but certainly doesn't address the kinds of things that I think you're concerned about from a bioterrorism standpoint.

And the other area that we're involved in is to continue efforts to optimize the logistics and transport operations.

And just as a summary of this, in terms of our plan, we maintain a six- to eight-week supply of blood bags in the U.S. We have three domestic warehouses and shipping points. We keep in transit in terms of blood bags from our manufacturing facility in Japan, four to six weeks for the product in transit via boat. We have three domestic trucking firms that we work with, plus DHL, FedEx, et cetera. We have computer back-up systems and so forth, although I've got to tell you, with that So Big virus, it slowed things down

quite a bit. But it didn't shut us down.

So that's really kind of the summary that I wanted to give you in terms of our view of blood resource availability. I think the take-home for me, I guess, or from my perspective is keep the system simple and provide a more centralized, if you will, leadership situation so that the donors get a clear message as to what's necessary and how they should respond.

Thanks for your attention.

DR. BRECHER: Thank you, Jeff.

Questions or comments? Andy?

DR. HEATON: This is Andrew Heaton, Chiron. Jeff, do you have a minimum lot requirement for maintenance in country? And do you have a lot release process as well?

MR. MIRIPOL: Oh, absolutely, yes. In fact, you saw it. To that point, with regard to the manufacturing safety or manufacturing processes, Terumo, Baxter, Pall--I'm sure we're all quite equivalent in this way--we have very extensive lot release quality assurance

requirements. And, actually, to get into our manufacturing facilities is very, very difficult. I think if we're talking about secure systems from soup to nuts, you have to have special IDs; it's all kind of--doors are controlled electronically, et cetera. This is both in the U.S. and in our manufacturing facility in Japan.

You know, we vet our employees to a reasonable extent. We're not doing, you know, CIA-type vetting of employees. But, you know, we have a very high level of quality assurance. Essentially, our philosophy is to have quality built into the product. Every blood bag, for instance, is inspected by special photo systems that, you know, ensure the labels are properly placed and needles are properly placed, et cetera, et cetera.

So I think from the standpoint--to your point, Andrew--of quality systems and assurance of the raw material that goes in the blood bags, the solutions and the bags themselves, that's an extremely high level.

DR. BRECHER: Lola?

DR. LOPES: I have a question about the margins that you mentioned. It seems that in an industry where there are few specialized manufacturers, you're making a critical product.

MR. MIRIPOL: You're right.

DR. LOPES: And it really doesn't cost very much in the context of, say, an open heart surgery, something like that.

MR. MIRIPOL: That's right.

DR. LOPES: Why are your margins small?

MR. MIRIPOL: Well, there is competition.

DR. LOPES: But you don't have many--this is not a commodity product.

MR. MIRIPOL: Well, in some folks' eyes--I look around at some of my folks that I supply. In some folks' eyes, they believe at least blood bags and certain types of products are almost a commodity product. I mean, it's a very good question. Obviously, we're not colluding with Pall or with Baxter in terms of trying to maintain prices. I wish I could because it would make

things a lot easier.

[Laughter.]

MR. MIRIPOL: Well, at least they'd be more stable. Let's put it that way.

You know, I think the real problem is this, as I see it: AdvoMed has kind of approached this. I know AABB, et cetera, have approached this in terms of reimbursement for blood products. And if you take that chain all the way down, you know--blood is kind of neglected as a product within, you know, our health care system. And so, really, it's a small percentage of the total cost of the medical care, which is one of the reasons why, you know, the payee system is really kind of ignoring it to a great degree, because it's kind of in the, you know, decimals or the dust of the total cost.

But, in reality, to a blood center and, of course, to us, it's a major part of, you know, the whole economic equation. But the problem is blood bags as a technology is a relatively old technology, and a lot of folks are in the business worldwide, although not in the U.S. And there are

some companies that are looking to get into the U.S. market, and, frankly, they may have a lower cost structure and believe that they can make some money in it.

DR. BRECHER: Jay?

DR. EPSTEIN: Dr. Lopes raised the issue of market concentration. I just would like to hear your thoughts. It seems as if the market forces favor concentration in many areas related to blood products, but that the security of the system actually favors more redundancy. And even where you have multiple manufacturers, you also have system incompatibilities. So, you know, the user really does end up committed, both through contract mechanisms and through system incongruities.

I just wonder what your thoughts are about the problem of lack of redundancy and how the market forces play out and whether there's anything that could be done in that situation.

MR. MIRIPOL: Right, right. Well, you know, to that point, actually if we're talking about blood bags themselves, although they're not

all identical, they are fairly interchangeable. If you start talking about things like apheresis kits and equipment, absolutely correct. I mean, you know, a Gambro kit will not work on a Baxter device, et cetera. And certainly our blood bags, you know, will work with everybody's centrifuges, as will Pall's and Baxter's and that kind of thing.

So I think from the products that are simpler, i.e., blood bags, they are more interchangeable, which may be, again, one of the reasons why the margins are lower, as opposed to the apheresis kinds of equipment.

I think that in many respects I actually would argue that the FDA requirements and the kind of oversight have been very effective and appropriate to have at least enough competition while at least providing a consistency of products. That is, I am not sure we want to have, you know, 20 different kinds of additive systems and, you know, blood that can be stored for 28, 35, 42, 49 days, because that can get more complex. Right now we basically have two red cell products: CPDA-1,

which is stored for 35 days, and additive systems which are stored for 42 days. But as you well know, probably--what?--85 percent of the blood in the U.S. is stored in additive systems, maybe even more than that.

So I would say that consistency in the types of products is appropriate, at least to the point that it doesn't stifle technology innovations or improvements, and that from my perspective, at least on the blood bag side, you know, having three suppliers gives the nation a fair security and redundancy that if, you know, the Terumo plant blows up or the Pall plant blows up, somebody else can step in and supply the product. Certainly we couldn't supply all the U.S. I don't think Pall can supply all the U.S. And I doubt that Baxter could supply all the U.S. right now.

Does that answer your question?

DR. EPSTEIN: It's helpful.

MR. HAAS: I was listening to the presentation, but the word "complex" I think fits here on multiple levels. We talk about market

forces, and that gets my ears perked up really quickly, and it's awfully hard for me to understand the word "market forces" at the same time we have reimbursement issues, which are directly counter to what a typical market force would be doing

The idea of a few sellers, typically, the idea is that, okay, that's bad because of potent--you know, that we can't collude, but yet there's a word in my discipline of "conscious parallelism," but in this type of marketplace you want the consistency. I agree with that term quite a bit; the whole sense of what is the relationship between the reimbursement and technology.

Well, there's an awful lot of literature that tells us that when reimbursement was rich and full, technology thrived in the medical industry, and now we have a situation where the reimbursement levels are constantly being ratcheted down, so the question becomes one of what's the impact of that in the future?

And then I think there's another issue that connects with this, too, in that here we're

talking about--I'll be unfair--but blood bags. We talked a lot yesterday about the blood products, and then we started thinking of plasma or components coming from the products, and then we think of the recombinant, which isn't a blood product, but it certainly is important in that area.

And then the question is, all right, what's the interaction among these? What happens if in one of these areas there is a problem? What's the spillover effect on all of the other areas? We have cascading effects in multiple issues.

And I guess, as I'm sitting here, I'm saying to myself, well, I can't pull this all together, but this is something I think that we need to be looking at.

MR. MIRIPO: Well, let me make a comment, if I may. You know one of the bad things about blood is that every individual unit is a separate lot. Every donor is different, but that's also, you could say, a good thing because, to your point

about, you know, like a blood product that was derived form a recombinant product, if you had a bad lot, you're really up a creek. You're talking about a lot of product that's bad, and that's happened with IBGG, et cetera. While if you have some blood bags or let's say blood in a bag that's bad, you've got one bad unit.

So you might argue that having blood as a product, in some sense, offers a lot of redundancy, as opposed to something that's made in a drug environment, where one lot is many, many doses. And if there's something wrong with that, you've lost many doses, and that could have a major impact.

MR. HAAS: A quick follow-up. I think you're absolutely right, but then that again triggers another thought in my head. If we think in terms of the recombinant market, for instance, if the hemophilia users were to go almost exclusively to recombinant, that affects the blood market in the sense that now the plasma products aren't being used to make clotting factors.

Does, and I'll say in quotes, the "market" then tell the blood sector to stop making clotting factor and then a clotting, a recombinant plant goes down, the suite goes down, then where's the back-up? And we just went through a situation like that, where, if the plasma, the Red Cross hadn't been there to step in, there would have been terrible problems. So, yes, I think this, we've got to look at this stuff.

MR. MIRIPO: Other questions?

DR. BIANCO: Jeff, that's been stimulating discussion, but you didn't talk much in terms of technology potential, in terms of that could facilitate availability. I have one that is my favorite, a needle that doesn't hurt.

[Laughter.]

DR. BIANCO: But other things--how do you see that facilitating or not availability, particularly if we want to build a reserve in the country?

MR. MIRIPO: You're right. I didn't spend a lot of time, in terms of new technologies

because, frankly, as I did point out, the things that we're doing are relatively low-tech, and I think the things that we're also doing don't really relate to sort of safety so much, except for perhaps the diversion bag, and you could certainly argue filters are helpful to some degree.

You know, I could, and we've had this discussion, argue that some of the things that are being proposed to improve blood safety will make availability worse; i.e., pathogen reduction technologies, et cetera. That, actually, even if they work, a platelet product is now less efficacious, and you've got to use more of it. So you've got to get more platelets, et cetera.

So one may see that some of these technologies could have a negative impact, and perhaps a substantial negative impact on blood availability. So it's the old story of, you know, unintended consequences. I think you need to think some of these things through, which is the reason I'm interested in, even though I'm no expert in, the bio sensor area. I think that that could be a

very interesting way of maybe assuring safety of the products in a simpler fashion.

And the real problem is getting the donors and getting the donors in the right place. So I think it comes down to kind of a better national program, perhaps, better coordination. As you well know, Celso, with your members, there are some members that don't appear to have a problem getting donors and others that do. Why is that?

Well, it's complex. It has to do with populations, and urban/nonurban, this, that, and the other thing. There's a lot of variations in that, but certainly there are some best practices that could, perhaps, help improve donor availability and donor collections.

I'm not sure if I answered your question, but it's a complex situation, and I think new technologies could have a negative impact on blood availability.

DR. BRECHER: Last question, Chris?

MR. HEALEY: Yes, I'd just like to echo what Paul said. I think it's completely

appropriate for the Committee to consider those factors, the relationship of the plasma therapies, and all of the ingredients that go into making those in the blood sector, and how those relationships work.

You pointed to the role of reimbursement, and I guess we're going to hear more about that this afternoon, and the importance of that to preserving the viability of the blood industry and the plasma industry. And, Jeff, you mentioned some of the regulatory issues and cost factors, and I think that's the other side of that coin, that also this Committee might want to consider is what are the cost factors that go into producing these therapies in these blood components, and how could those costs be managed better. Because, obviously, you have the cost, and you have the reimbursement side, and those need to balance out to make the industries healthy.

MR. MIRIPO: Right. Thank you.

DR. BRECHER: Thank you.

Our next speaker is Jerry Holmberg from

Haemonetics.

DR. HOLMBERG: While things are getting set up, I just need to make a point of clarification. When Mac asked me to speak, I had to quickly tell him that I was no longer employed by Haemonetics. So please bear that in mind, when I go through my presentation, that I do not represent Haemonetics. I will be showing you a few slides, with their permission. However, I've been involved with frozen blood, and especially the ACP-215, for many, many years.

By the way, can Drs. Haas and Penner identify? Okay.

Dr. Haas, I just wanted to tell you that I send greetings to my schools, my alumni. I did a master's at Michigan State, and I will be talking a little bit about my experience at Michigan State years ago back in the '70s, and Bowling Green, I obtained my Ph.D. So I haven't been back to either institution for a long time, but one of these days I'll get back there.

I was invited back for the medical

technology reunion a few years ago and was unable to attend that due to my son's wedding.

DR. PENNER: We won't forget you.

DR. HOLMBERG: Thank you.

DR. BRECHER: See, that gets to the point that the alumni offices can always track you down.

DR. PENNER: Oh, yeah.

DR. HOLMBERG: What I'd like to do today is to go through some of my experience, both in the civilian world and in the military world. After I left academia and I decided that what I wanted to do was to get a little bit more management experience. So I went into the military, and, boy, did I get management experience. So I went into the military relatively late in life and recently retired about three years ago and joined Haemonetics at that time.

One of the things that I want to talk about, first of all, I want you to keep in mind, as I go through this presentation, because I will be giving you an historical presentation on the history of frozen blood and where we are today with

some new technologies.

But two things I want you to keep in mind, especially as you look at the task force for reserve of blood and even of frozen reserve, and that is--and I learned this from the military--keep it simple and practice every day like you're going to war. Okay? I would tell my people, and I ran one of those facilities in Okinawa, Japan, that had 40 freezers and 40 M115s, and we practiced on a quarterly basis with a disaster drill activation of my augmentation teams, and we also integrated frozen blood on a daily basis. So just keep those two premises in mind.

Yesterday, I think you heard that frozen blood has been around for three decades, but really it's been around longer than that. Dr. Valeri actually has done or did frozen blood transfusions back in Vietnam, back in the '60s. It's been primarily used in this country as a source for rare cells.

And one of the things Dr. Valeri and I have great philosophical discussions on, and I

think that where we might be missing the boat is that it is a strategic, a quarantine strategic reserve. There are places around the world that continue to suffer with high rates of HIV, and blood tends to still be a vector for that transmission.

Whether that's feasible to supply that to those Third World developing countries is beyond my financial means and also I think a lot of other supporting World Health Organizations or whatever. But it is a quarantine strategic reserve.

It's also been looked at for many, many years as a strategic source for high-quality red cells, such as red cells high in ATP and 2-3 DPG. This is one of the reasons why you notice in yesterday's presentations they talked about--Commander Bartley mentioned about six-day-old blood. Routinely, six-day-old blood is frozen, and if you do go on for expiration and rejuvenate the whole thing that you're trying, the target that you're trying to do is to regenerate the ATP and the 2-3 DPG levels.

Forty years ago, there was also, and I put this, I want you to understand this was 40 years ago that deglycerolized red cells were viewed as washed cells to remove the plasma, also to remove white cells because they will reduce white cells down to a two-log reduction. It doesn't meet our current standards today, and so therefore that claim cannot be held.

But also back in the '70s, it was thought to really, it was thought maybe this was the blood that would be free of hepatitis. And what we did find was that it did reduce viral load, but it did not eliminate the risk for those--for hepatitis. And then also CMV, many of us years ago used to use CMV as a frozen blood, deglycerolized blood, as a source of CMV-free cells.

Some of the historical disadvantages of using frozen blood is that it's been time-consuming, very costly. Up to now, it's been limited to 24 hours due to the open system. And while it reduces the viral load, it does not eliminate the viral risk. And also we have new

required tests that have become available. So, therefore, it does require that a cryo sample is maintained to be able to retest those units.

A little historic graph here is that frozen blood really became popular in the '70s, as far as a lot of hospitals, local hospitals, blood centers using it. I refer back to my days at Michigan State, back in the '70s, and our small community of Lansing and East Lansing, Michigan. We had the American Red Cross with the frozen red cell program, and Ingham Medical Center and also E.W. Sparrow Hospital. So three locations in that one small area had frozen blood available.

What happened, though, was towards the end of the '80s, when the DRGs started kicking in, the hospitals found that this was very costly, and they could not really support the cost of a frozen blood program.

You see my graph continues to go up because in the early '80s the military really got into a process of thinking about, and what the document was called, it was called Military Blood

Program 2004. What would the blood community look like in 2004? Well, guys, we're one year away. And one of the things that the military really pushed back then was that we needed to have a frozen reserve available--pre-positioned, pre-tested in strategic locations throughout the world.

Also, throw in there that we have the GMPs. GMPs came on in the early '90s, and that may have had some effect on the popularity of using frozen blood, also the Gulf War. The Gulf War, we had a lot of frozen blood available, but one of the things was that it was not used that much because we had plenty of time, and the liquid blood supply was able to be put into place.

Then, 9/11 kicks in, 2001, and then we start seeing another surge in interest with the frozen blood, and both the military and the American Red Cross and NIH have looked very seriously and have purchased equipment to try to move for a closed system.

Today, if you look at the frozen blood that's available. About 38 percent worldwide of

red cells, rare red cells, of the frozen blood inventory poses 38-percent rare cells, about 60 percent is a reserve by the military, and that's not only the U.S. military, but other NATO countries such as Belgium and the Netherlands, and then 2 percent is actually autologous.

So is the concept of strategic frozen blood reserves still viable after 30 years? Well, my answer to that is, yes, but re-engineering is needed. I think in the conversion of moving my slide to the hide drive here, it sort of got messed up, and you see some of my points early, but let me just walk you through some of my points.

Re-engineering was needed. Boy, putting in a hard drive really messed this up, so bear with me.

You need to be able to freeze all anticoagulants and additive solutions. Currently, the device that has been cleared, which was cleared back in May of 2001 by the Food and Drug Administration, was a closed system for Haemonetics based on CPDA-1 red cells frozen within six days

and also stored at minus 80 degrees Centigrade.

Subsequently, upon thawing, those cells are washed and then an additive solution, AS-3 is added to them, and because it's a closed system, it would be 14 days.

The current problem is that the requirements that the company built the device on, and also did the clinical studies, was based on what the military was using at that time. And the military was using CPDA-1.

Now, this is where I have a disagreement, and again this is my personal view, and please take it as personal view, but this is my personal view as a problem that is currently being brought before the FDA is that, once you take a unit of red cells that are six days old and you remove the additive solution, and you add glycerol through a process to get to a 40 percent, then that's a glycerolized red cell. And yet there has been a requirement to go back and to test all anticoagulants and all additive solutions to ensure that the quality is there and also that the survival times are

comparable. So that's where the manufacturers are at the present time--are going back to qualify the other anticoagulants and additive solutions.

Another issue that is currently being brought is the whole issue of temperature because of the original studies were presented to the FDA at minus 80, however, the CFR and the American Association of Blood Bank standards say minus 65 or colder. And so the FDA is requiring the manufacturer to go back and to qualify that temperature, also.

We also need a closed system, which the new system that I'll show you in a few minutes does. A closed system, from the very beginning, from adding the glycerol to removing the glycerol, it needs to be automated, it needs to be simplified for minimal training. Again, keep the kiss system in place, keep it as simple as possible. Reduce the solutions.

One of the things that we learned in the military was that, you know, when you're aboard ship, you can't have a lot of fluids, and the whole

top deck of the two hospital ships is primarily for fluids, for supplies. And so if you can reduce the amount of solutions that you have, you can move your army or your sailors better.

The storage conditions. I already mentioned mechanical freezers. The CFR and the AABB currently say minus 65 or colder, but the hang-up right now with this closed system is minus 80.

Reduce breakage. Dr. Valeri had an excellent article this last I think it was March, in Transfusion, that showed, with the Merryman method, with the stericon bag, that the breakage was very high, as opposed to the PVC, and Dr. Valeri strongly recommends the PVC bag, the Baxter PVC bag, that is qualified at minus 80.

One of the things that I would, and I'll show you in some of my recommendations, is that when you have extended storage, you reduce the need to be able or to have to move frozen blood. You, the logistics change, the logistics now change to a deglycerolized red cell that you could ship as you

normally would ship a unit of packed red cells or whole blood at 4 degrees or a temperature of 1 to 10 degrees Centigrade in the shipping.

The extended dating for the post-thawed shelf life. Again, with AS-3, documentation has been 14 days, although there have been studies that show that could even go out to 21 days or even longer.

And then my pet peeve is that many times when people have frozen reserves, they ignore this last principle, and that is integrating it into normal, routine use. If you integrate it in, you get the hospitals, you get the physicians, you get the technicians used to using it. This is the ACP-215, 56 pounds. It looks like very many of the other devices that Haemonetics has on the market.

On the left-hand side is a shaker, and on the right-hand side is a printer that will record all of the critical information that takes place during the process. It tries to keep in mind all of the requirements for the GMP and record those data points.

This is a set-up with the glycerol. The glycerol is hanging on the side, and it gets mixed to what the military uses as a primary collection bag, and this would be the 800 ML PVC bag.

I know yesterday there were a few questions on the productivity for glycerolization--the 215 well-glycerolized red cells within 11 minutes. You could do four units per hour, and the only solution that you use is glycerol. Now, the thing that you do not understand here and is not clear from the picture is that I mentioned in one of my earlier slides that the requirement was to reduce solutions, and in order to reduce the solutions, again, trying to make things simplified on the other end, when you want to deglyce the unit of red cells is that you need to remove the supernatant glycerol. So there is an additional step here of spinning the red cells down, removing the supernatant glycerol and getting the hematocrit back to a 60-percent volume.

If you reduce that supernatant glycerol, then, on the other side, when you deglyce, then you

can use only two solutions, the 12-percent sodium chloride, and the .9 .2-percent sodium chloride to remove the glycerol and then your AS-3 for an additive solution.

The procedure time for deglycerolization is about 45 to 60 minutes, depending on the size of the unit. A larger unit will be going a little bit longer. It takes I think, for a rough estimate, we heard this yesterday about one hour. There's one kit involved, and there's three solutions--re-agents, I should say.

I apologize for the color on this. It was all black, and I don't know what happened in the transfer, but the protocol, if we look at an 8-hour shift, you could glycerolize 20 to 24 units. To deglyce one person per machine, and that's just, I should just say one machine is 20 to 24 and 5 to 6. For 16 hours, you could go 40 to 48 for glycerolization and deglyce 10 to 12.

Now, as was mentioned yesterday, if you had multiple devices, one person could operate quite a few devices. And the process is that if

it's going to take 45 minutes to 60 minutes to run, you could set it up, start it, and move on to the next device and set the process going.

So let me ask you, strategic reserve, is it a pipe dream or is it reality? And one of the things I just would like to add, and again please remember that my comments are my comments and not recommendations from anybody else, other than Jerry Holmberg, but I would have to say, as far as the task force, the comments that Karen mentioned yesterday, I think that a better approach would be to have a, I shouldn't bipartisan, but a pluralistic approach, I should say a pluralistic approach to the reserve, a pluralistic approach being liquid blood and also frozen blood.

When you have an emergency, you don't want to be collecting blood at that time because it's going to take you hours, days to get it completely tested. You don't want to, you want to be able to immediately get the blood off your shelf and get it to where it's needed.

The frozen blood, as was mentioned

yesterday by Commander Bartley, is a stop gap, and it is to back-fill the needs of the local facilities. I think strategic reserves are necessary for our seasonal shortages, such as holidays. We all, this last year, we had some, here in the D.C. area, had some tremendous snowstorms. I bought a snow-blower the 1st of February, and I used it for three major snowstorms, and I thought I left that when I left Bowling Green and Michigan State.

And by the way, I think Bowling Green is worse than Michigan State, as far as the amount of snowfall because at Bowling Green you get the drifting.

[Laughter.]

DR. HOLMBERG: You know, illnesses we all face ever year, we face the colds and the flus and things that come up. So I think that strategic reserve is a great way to sort of take out the valleys in our blood supply.

I think that it's needed for both local and national disasters. I hear all the time people

saying, well, you know, in Oklahoma City or in Kansas City or in New York, New York, with the Trade Centers, you know, they didn't really use that much blood because of the casualties. Well, you know, that's unfortunate, and big disasters like that, yes, there's going to be a lot of casualties. But from time to time there is going to be that local need for a surge in the blood supply, and there's going to be a surge--there's going to be a need to backfill that surge.

The strategic reserve I believe is needed, again, for homeland security, in addition to local and national disasters, and also as we face this year emergent pathogens such as SARS--and West Nile has been with us for a few years--I think it would help even in taking care of those kinds of situations.

Again, my personal opinion, but what is needed? I think we need a strategic national plan. And this may be heresy to my colleague sitting over here in the blue uniform, but I strongly believe that there should be a strategic complement to the

military system. The military system, we need to have a ready reserve within the military at all times. That does not mean that from time to time the civilian sector, which all of us are taxpayers, could not, should not be hindered from going to that reserve and saying, hey, look, we need blood, let's move some of the inventory and work that.

The whole idea is keeping it going, making sure that the total defenses of our country are met, and the first priority is to the military, but I say secondary responsibility is to the civilian sector.

We need strategic locations. I don't think that we're talking just one or two locations. We're not talking the military has two locations, one outside of San Francisco, the other one at Maguire in New Jersey, which is Philadelphia and New York City. I think that 16 is probably unmanageable, but I think that some strategic locations with a workable number, on the coasts, on both coasts, and also in the mid would be very beneficial.

I also agree with Dr. Gilcher, and that is that the reserve--I think we should look towards Group O blood and having those reserves available for and eliminating any potential problems in a disaster of Group O mismatches and just work with the Group Os, both positive and negative.

The next comment that I would like to raise, as was mentioned the other day, the 10,000 units came up as a target level. That was sort of--I think that was speculation or just a guess. But we do need to come up with target levels. What could we be able to have available, both liquid and frozen, to be able to move to strategic locations? And remember again that in 9/11, we did not have our air traffic. And that created a major problem. So we have to make sure that we do have target levels and we also have strategic locations set up.

Also, I would strongly recommend that instead of sitting on a frozen reserve and let it just sit there, I think that you need to constantly be rotating it and replenishing it. I think one of the problems that the DOD had early on was that

they collected a large bolus of blood back in the early 1990s for frozen inventory and then the ten years came and without the subsequent replacement. And I think we all know that criticism there, but it's an idea that you have to constantly be replacing your frozen inventory.

So I'll conclude with questions that you might have for me.

DR. PENNER: Jerry, just a quick question, because this hasn't been handled, but I'm presuming the information is there. Red cell survival in the frozen products now with the present additives is comparable to what we would see in most cases with the liquid blood, and the other thing would be the oxygen delivery of the frozen blood once reconstituted.

DR. HOLMBERG: Well, as far as--the FDA does have a requirement for red cell survival, and that must be the 70 percent or 75 percent--the 75 percent. And so, you know, there will be a little bit lower red cell survival than with fresh blood, if you were using fresh blood.

Now, as far as--but, still, any anticoagulant or additive solution has to meet that requirement for the expiration period of time. The 2,3-DPG and the ATP levels, if you collect this at day six and you freeze those red cells, the levels of ATP and 2,3-DPG are maintained at those--in the frozen state.

DR. PENNER: As I recall, though, the 70 percent, is that 24 hours, wasn't it?

DR. HOLMBERG: Seventy-five percent, 24 hours later, 75 percent of the red cells must be in circulation.

DR. PENNER: We never really checked the survival time, half-life, 25 days, something like that, so--and that data is around someplace, but it never was focused on as whether the cells really survived 48 hours or 72 hours.

DR. HOLMBERG: That's a good point, and I would direct you to the article by Dr. Valeri. It appeared July 2001 in Transfusion, and I know that the most recent study that the American Red Cross and Haemonetics is doing is looking specifically at

that, not so much from an FDA requirement but for publication purposes to be able to put that in a publication.

To answer your question, I don't have that data right off the top of my head.

DR. PENNER: Okay. And maybe one other question if I can. That is, outdating on a contingency, what would your feeling be to extend outdating for liquid blood under certain circumstances, an emergency use, to 47 days, to 50 days, to 60 days? We know that at 42 days everything doesn't dissolve, that the red cells are still red cells, and we've got plenty of data that show that if you extend it on out, you lose something. But you don't lose everything. You still have an oxygen delivery system.

DR. HOLMBERG: Well, I would caution you there, and you just hit a nerve with me; that is, I think it's wrong to manage an inventory based on 42 days, primarily because although the ATP levels--or I should say although the 2,3-DPG levels will come back within 24 to 48 hours to the normal level, the

problem is that if you have an acute situation and you have trauma patients, do you really want to be pumping them with that kind of blood, or do you want to give them fresher blood that has more readily available oxygen offload capability?

And so my question is that, again, I would frown against going beyond the 42 days, extending that. I think that we need to manage our donors and manage our inventories so that we're working with a shorter inventory.

DR. BRECHER: Andy?

DR. HEATON: Yes, I have some comments on blood storage. The new additives have very high post-transfusion recoveries of 42 days. They run about 85 percent, between 80 and 85 percent. And as for the DPG issue, DPG has a half-life, once you collect the unit, of about ten days. So at six days you've lost 50 percent of your 2,3-DPG. The reality is it's regenerated with a half-life of six hours when it's been transfused, and there's absolutely no evidence that long-stored blood doesn't transmit oxygen. There are many other

factors of oxygen offloading and onloading that would suggest that even long-dated additive stored blood will be quite effective, even in a casualty situation.

So I don't see that there's a quality issue here. It's really a matter of just recording the integration of the new additives with the old frozen storage solutions and getting that on the record.

DR. BRECHER: Jeanne?

DR. LINDEN: Jerry, could you educate as to what's involved with the validation of the 14-day shelf life for the other additives and temperature conditions and so forth? And did I understand you correctly that such studies are underway for the presently used additive solutions?

DR. HOLMBERG: Yes. Studies have just been completed for AS-1 and AS-3 at the various temperature ranges. The AS-5 is currently in the process, waiting on the military, and the funding for that, I believe, to study that.

What is required to complete those studies

has been to be able to prove at two different temperature ranges, at both a 70 plus or minus 5 and at an 80 plus or minus 5, to make sure that the range of, you know, minus 65 or colder based on the minus 80 is equivalent and that there is no problem with that. Also, in-vivo survivals are required along with red cell quality parameters.

I think that answers your question.

DR. BRECHER: Jay?

DR. EPSTEIN: Are you also studying use of rejuvenation solutions to permit delayed freezing with the system?

DR. HOLMBERG: Currently I am not. I know that the Red Cross is looking at a pilot study, but I can't elaborate any more on that.

DR. BRECHER: Keith?

DR. HOOTS: Just a logistical question in terms of disaster relief and that sort of thing. You had suggested that you'd use frozen supply to replenish, just as the military proposed, the acute liquid supply that was used for the immediate management. How do you foresee the coordination to

occur? For instance, if there's a major disaster in New York, and Philadelphia immediately ships X percent of their available supply to New York to provide the second wave of supply, who should be responsible then for freeing up the frozen supply to replenish Philadelphia? That's one of the things I guess, when you're talking about how to fit this whole thing together, is this something where it would just be--you would just requisition it because there was a need? Or should there be some supervisory planning group that says, okay, fine, can Philadelphia release X thousand units and, therefore, we immediately within 24 hours could use the frozen supply?

DR. HOLMBERG: Well, I agree with you. I think that there needs to be a guidance, some sort of group or consortium coming together, whether that is mandated by the government or just on the volunteer or the blood agencies out there. And I think the first big step is the task force that Karen mentioned. And I think that it would have to be worked out with agreements. Ideally, if

Philadelphia had their own frozen inventory, they would dip into that and start replacing that.

Now, as far as who covers the cost, I think we need to work--those are issues that need to still be worked out.

DR. HOOTS: But I understood you to say that you thought 16 depot sites were probably too many. So, logistically, you might be talking about eight around the U.S. So, presumably, if you really were talking about a depot system, then Philadelphia might be a site, but in that sense it wouldn't belong just to Philadelphia.

DR. HOLMBERG: No. And, again, that goes along with my concept of the civilian and the military working together.

You know, I would view a kind of a concept like that that it would actually be--it would really be--the primary goal, the primary objective is our armed forces, but secondary would be our own internal security. And working out of that inventory, and whether it's, you know, six places or eight places, there may be a requirement to

backfill Philadelphia with liquid blood for a period of time until they could get their deglyce done.

But I firmly believe that during a disaster is also not the time to be collecting donors. I think you need to be redirecting some of your energy in other locations.

COLONEL SYLVESTER: I'd just like to make a comment. I think some of what Jerry is talking about and other people alluded to already exists. There is much communication between the military and the AABB, the ABC, the ARC, and we have at times, when necessary, shared our inventory. We purchase from them, and then we'll turn around and supply them when we have excess and they're short. That occurs now. So I don't want the panel to think that our inventory is never touched by anybody else. We do rotate our inventories in and out and share them when necessary, as happened after September 11th, because we did have the ASWA at Maguire, which was driving distance to New York City. We were ready to push whatever was needed at

Maguire forward into New York City. It just wasn't needed. But it was stationed there and ready to be put on, and we do the work through the task force and we do the work informally in our coordination with the other agencies.

DR. BRECHER: If there are no other questions or comments, we'll take a break until 10:15.

[Recess.]

DR. BRECHER: Okay, if we can take our seats?

We're going to allow one other company rep to make a comment. Steve Binion I believe wanted to make a comment from the previous talks from Baxter.

MR. BINION: Thank you, Dr. Brecher and the Committee. Just very quickly, two comments as a follow-up to the industry comments earlier.

I did want to just bring to the Committee's attention that from the standpoint of new technologies related to potential improvement in availability of blood products, in addition to

apheresis systems for collection of red cells on the market by other manufacturers, Baxter does produce an ALYX apheresis system. This is a mobile system which is available for simultaneous collection of two units of leukoreduced red cells, with a total collection time of 20 to 30 minutes.

And then just a quick follow-up to the commentary regarding blood pack unit manufacturing capabilities and supply, Baxter does maintain multiple redundant manufacturing plants and capabilities, and, in fact, our total capacity is sufficient to supply the current U.S. market requirements in the range of 15 to 20 million blood pack units per year.

So I just wanted to add those comments to the record from the standpoint of manufacturers. Thanks.

DR. BRECHER: Okay. Thank you, Steve.

We'll now move into the public comment portion. Are there any public comments?

[No response.]

DR. BRECHER: If not, we're going to move

to the--oh, I'm sorry.

MS. O'DAY: I'm Miriam O'Day, and I'm here on behalf of the alpha one community and the immune deficient community.

DR. BRECHER: Pardon me, ma'am. Just a minute. Is this about the HOPPS thing or--

MS. O'DAY: It is.

DR. BRECHER: Okay. We're not there yet.

MS. O'DAY: Okay.

DR. BRECHER: We're going to talk about HOPPS a little bit later. We just want to do the discussion from what's already happened.

MS. O'DAY: Okay. My apologies.

DR. BRECHER: Then we'll open it up again.

MS. O'DAY: Thank you.

DR. BRECHER: Okay. If there are no comments about the previous discussions--I'm sorry I didn't clarify that--what I'd like to do is discuss two things. One is what has already ben discussed or presented in the meeting about supply, reserves, et cetera, and there's already been one motion that there may not be any resolutions we

want to make. And then I want to just move on to an update over the question of the Dr. Carmona letter and the question of recombinant factors.

Let's just first deal with the question of do we need to have a resolution or do we just need to take this as information and then go on to other meetings to expand and get further feedback from the interorganizational task force as to what their status is, et cetera. Mark?

MR. SKINNER: During the brief Committee discussion earlier, I had two questions I wanted to ask, and the second one I didn't ask. And it came up, I think, in two different presentations yesterday, and I think it's a piece of information that at least would be helpful to me as a consumer.

The discussion was around when there are new tests that are developed after the products are frozen. I think the statement was made that, with the authorization of the physician, they could use a non-tested product, like when the West Nile virus was implemented. I didn't hear anywhere in that context under what conditions the physician can use

it, if it was emergency only, or if the consumer was ever informed that they were using it, if it could be used on a routine basis, you know, after you had given your general assent. But I think we need to probe that area a little bit if we're using products that have not been tested with the latest tests.

DR. BRECHER: Anybody want to respond? Celso?

DR. BIANCO: I can try, Mark. There are routines established in all blood centers, collecting facilities, that involve an emergency release, and this occurs regularly with an extremely rare unit of blood that is being stored for many years when other tests come. And it's going to be--there are several levels. The first level is obviously the notification and acceptance of that unit by the hospital and the physician that needs that unit. And that physician, in conjunction with the patient, will obtain an informed consent, will then discuss. Frequently it's not even a question of informed consent about

the patient. Those units are used in extremis. And so it's the family of the patient actually that ultimately will be--it's like a life-and-death decision that will lead to something like that.

But, actually, in all FDA guidances that you will find in all materials, there is always a small chapter, a small paragraph that will discuss what kind of labeling--that unit that doesn't conform to the current specifications, how it should be labeled and what the requirements for an emergency release.

DR. BRECHER: Karen?

MS. LIPTON: I also just wanted to add that under the AABB standards there's a section on it called emergency release for what we call non-conforming product, and that would require that you get the--that you inform the physician. Now, you know, as to whether the physician goes back and says something to the patient is really between the physician and the patient. In most cases, though, once you go to emergency release, the physicians aren't accepting the blood, anyhow. So it's--you

know, it would really have to be dire circumstances before they would do that.

I think your question, though, Mark, also goes to this question of how do you rotate inventory when we're introducing a new test. And that is generally something when we're introducing a new test that we do discuss with the FDA, and a lot depends on really the significance of the test, whether you would go back and test all of your inventory. There are different circumstances with West Nile. I think when we were talking about not so much the testing but even some of the questions, some of the issues related to whether there really was any different risk as of the day we started testing or not. But testing inventory is generally--it actually is something that's always addressed as a new test comes up as to whether that's something that needs to be done.

DR. BIANCO: And, actually, just to complete what Karen said, very appropriately, it is that for the most important tests introduced in the last 15 years, I would say, there was always an

attempt by the testing facilities to test them within the first day or two, notify the hospitals to hold their inventories, not to transfuse during that period until they could replace the inventory in the hospital with a tested inventory.

MR. SKINNER: I think just the issue that I was trying to get to was whether there was a difference between an emergency situation or whether they could be routinely put back into the supply with just the doctor's assent. And it sounds like what we're talking about is the emergency situation. So that wasn't clarified in either of the comments yesterday, and maybe that was just information that was assumed. But I wanted to--

DR. BRECHER: Yes, West Nile virus testing, of course, has been somewhat of an exception because it was a seasonal illness, and so it wasn't felt that we had to go back and test the prior inventory. But I can tell you, for example, we had a patient with six antibodies. We searched the whole country for units of blood, and the

patient needed a liver transplant. And some of the only units we could identify in the country were collected last summer and were not tested for West Nile virus.

We were prepared to use those if we needed to. The patient actually didn't make it long enough to get the liver transplant. But it was an extreme situation.

Other questions or comments? Is it the Committee's feeling that we do not need to have a resolution that comes out of the presentations made so far in this meeting?

DR. BIANCO: I may disagree with Karen that we don't need--it's not necessary--it doesn't have to be called a resolution, but I think that marching orders to the disaster task force to address the issue and come to us within a certain reasonable period of time.

MS. LIPTON: We could certainly do that. I mean, they're going to do it no matter what this Committee does. So if they'd like to say we encourage your--you know, support your efforts, but

it was--it's in the process, and it's not going to stop.

DR. BRECHER: Right, and we anticipate an update in the January meeting.

Yes, Chris?

MR. HEALEY: I'm neither in favor nor opposed to a resolution per se, but the one comment that I heard repeatedly yesterday was the need for Federal funding for a reserve if there indeed is one created. So that might be the basis upon which to carry something.

DR. BRECHER: Well, I think until we have a firm plan as to what kind of reserve, I think that may be premature. I think that would be the intent of the Committee, but we need to know exactly what it is that we want to encourage.

Okay. Mat?

DR. KUHNERT: Being new to the Committee, I saw there was a history of this issue being discussed in the past and that there was in the past a need to develop systems for monitoring, and now it seems there are multiple systems for

monitoring. So the only thing that I think would be wise to urge is to have one system, or at least if there are different systems, that they have clearly different purposes. So I'm not sure if that's something the Committee can do to urge, but it just seems with all these different systems there is some need to integrate them.

DR. BRECHER: Mac?

CAPTAIN McMURTRY: If I may, I'm not really prepared to talk about the monitoring. There's stuff going on. I just wanted to let you know that the idea that had been out there for a while, which was to look at the current DHHS system to have it analyzed to see how accurate it was, how relevant it was, that analysis is ongoing. We have gotten a couple of the deliverables. Those are sort of ricocheting around in the Department right now. No action has been taken, but we're anticipating it. But it's a little premature to be saying what that action is going to be. But the thought is to consolidate what the Department has.

DR. BRECHER: Maybe we can ask for an

update as to what the status is at the next meeting.

CAPTAIN McMURTRY: It would be mature at that point.

DR. BRECHER: John?

DR. PENNER: I agree, just from political aspects, that maybe not making a statement until we're ready to come up with something solid and concrete would be better than delivering a lot of small resolutions along the way, which then will be diluted out.

DR. BRECHER: And we can set a precedent that we don't have to have a resolution.

Lola?

DR. LOPES: I see a connection between the monitoring and the problem that was discussed a little yesterday of identification of individual units. It seems like if we can get to the point where units are bar coded, that monitoring could be linked in a single system so that it would occur automatically any time a unit moved from inventory to another place, came into the system, it would

all be done at once.

I think this is something that is worth hard consideration because I think that that in some ways might be simpler to do than the kind of monitoring that we do with human reports.

DR. BRECHER: We have a very fragmented system, and it's a huge system. There are some countries that do a much better job of tracking that maybe we can get some further information about in the future. But they tend to be a fraction of the size of our system.

Karen?

MS. LIPTON: I wanted to mention that all the units--they are bar coded, but I think what you're talking about is a single, and there is a system that has been implemented in some places, but not all, and it's the 128 ISBT, which would assign a unique code to every single unit and product. And that is something that has--it's been on hold. It is not as simple as you could imagine, because to make something that would work for everyone would require everyone's computer systems

to be able to handle this. And that is, frankly, the huge bar to this because changing a computer system in a blood center is--it's a very regulated function, and it's something that just--it just takes time.

So we're aware of it. I think it's something that could contribute to it. It's a huge financial commitment. It's also from many blood centers requires a tremendous commitment of resources to change. And we've said this before. This is a very fragile industry, and we don't just have--you know, you pick your priorities. And I think the sad truth right now is that this is not the top priority given some of the other things. If we had a different reimbursement system, if we had something that said this was very important, it could work.

So, as I said, I think it's out there. I think it's a matter of resources, and it's a matter of priorities.

DR. BRECHER: That was a good choice of words: "a bar to bar coding."

Mac?

CAPTAIN McMURTRY: I'd like to comment once again on the monitoring issue. As Karen said, it's a very fragmented system, and what we have found, when you go from a pilot test of nine sites to 29 sites to 5,000 hospitals, and then another however many community blood collection centers there are, the computer systems are so different across the spectrum that coming up with any kind of a monitoring system is going to be very difficult.

DR. BRECHER: Well, it certainly wouldn't be an automated system. You probably would have to input at the Internet level daily.

Jay?

DR. EPSTEIN: I just want to come back to the issue of making recommendations. I tend to agree that I may be premature for us to make additional recommendations at this meeting, especially given the history that we've repeatedly made recommendations in the area.

On the other hand, I think that there's a core issue where the Committee could provide a

useful service to the Department by coming to closure, and that's on the question of the need for what Jerry Holmberg called a strategic national plan. And I think it's one thing to call for, you know, studies of the issue and to call for support for development, but the central question, it seems to me, is: Do we want/need a strategic national plan? And what is the government's role in that?

I think that it's a bigger question than the way we've approached the issue in the past. There have been many times when the question has been raised who should manage blood in emergencies. I remember more than a decade ago that the same question was asked in the development of what is currently the FEMA plan, and, you know, we've revisited it in the wake of various natural disasters, and then we've been revisiting it in the mind-set of counterterrorism.

And each time the answer has come around to, well, industry can take care of this. And that's okay, but I think that what we need to ask is: Are the right things happening? And is there

a more directive role needed by government? Or even is it just a question of financing?

And I think that the role of government in this remains unclarified, the necessary role of government in this remains unclarified. And I think that although the existing efforts within the AABB Interorganizational Task Force are notable and commendable, it bears some examination whether we're getting the thing that we want done. And what are the obstacles? And is there a barrier based on the current relationship with the Federal Government? In other words, are there some things missing that should be there?

So I just kind of think that what we're really dancing around is a core issue, which is the role of government, and that we need to come to a point of advising on that.

DR. BRECHER: Karen?

MS. LIPTON: I agree with you, Jay. I don't think the time is now, though, because I think for us to make recommendations about the role of government or not, when we don't even understand

what we're dealing with. I do think that at a point when, you know, private industry could come back and say, well, this is what we've come up with, and then you can say, all right, what are the whole--you know, is there a better way to do this? Will this satisfy? What are the purposes?

I think we need something concrete to deal with, and, you know, to sort of start right now and to make a suggestion, well, we think someone should study national strategic reserves would frankly make me--or a national strategic plan would make me very nervous, because it has to be, you know, built on a system that we know we can handle. And at least when we come back from the disaster task force--and we're going to be very honest about what we think the barriers are. And if some of the barriers are we're too fragmented and somebody needs to step in here, I think it will be pretty apparent.

DR. BRECHER: Jay?

DR. EPSTEIN: Well, I wasn't trying to advocate recommendations now, so I agree with you,

Karen. But I think the implication of what you're saying is that the Committee ought to ask the AABB Interorganizational Task Force to come back and present its current thinking.

DR. BRECHER: Right. We have already said that they were going to--you may have been out of the room when we did that.

I think Jay raises an interesting question about what is the role of government, and maybe it's a bigger question, not just for emergencies but what should the role of government be in managing the blood supply on a daily basis. And that may be something the Committee could visit at some point.

Celso?

DR. BIANCO: I certainly--I want to express a certain concern about the question. I think that the difficulties that we have been having in shortages and issues of this kind, they exist. And I think that many of the reviews and studies that have been done have shown that the entire system is underfinanced. It is not a

question of management of the system.

Whenever we find the resources, we get the donors, and actually I must say that while some areas cry a lot that we don't have the blood, we don't have the blood, but we recovered even from the substantial donor loss that we had since last year with multiple deferrals, including the vCJD deferrals, that they total about 10 percent of our donor base. And it was very serious.

We have been able to address shortages that were caused by natural disasters and other disasters, and we hear Dr. Gilcher every time, beautiful systems and the beautiful ways he has been able to deal with it.

Yes, I'd like it to be discussed, but I'd like us to think of the role of government as a supportive, probably some coordination, facilitating communication, but mostly supportive. I think that the blood banking community has shown that they dealt with a lot of difficult situations, and with that, the patients have blood.

DR. BRECHER: Paul?

DR. HAAS: I think the question of private sector versus government, as always, is very difficult. I hope I'm not misconstruing your words, Karen, but having industry decide what it can do and then ask where the help is is a different question than saying there's an interactive relationship and we start out the process thinking in terms of where the interaction takes place.

I don't know how you start that. Someone has got to come up with the first step, which is maybe what the task force is doing. But if government is always what comes in to plug the holes, the probability, I think, is that the government will do that poorly because it wasn't part of the integrated approach to things. And so somehow we have got to be meshing the two constantly.

DR. BRECHER: Karen, does government have a seat in the Interorganizational Task Force?

MS. LIPTON: Yes. The FDA and the CDC are on tier one.

DR. EPSTEIN: And the Department.

MS. LIPTON: Excuse me?

DR. EPSTEIN: And the Department.

MS. LIPTON: And the Department, that's right. HHS, Jerry Hauer. So there is definitely an opportunity for the government to play a key role in this as we deliberate in terms of what we think the roles and responsibilities and needs for the government.

I mean, the one thing I will tell you is that, having worked with HHS during these times of deliberation, they're not so much in a command and control mode right now. They want systems to work. But everybody's got their hands full, and I think they're really looking for and working with a lot of private-government partnerships. It's more along the FEMA line, you know, where the rubber hits the road is in the local communities, not sitting up in Washington, D.C.

I think, again, I would encourage us as a first step to let this group come back and have HHS and FDA and CDC sitting at the table, and then if

it just doesn't look rational or if we want to pass it on up to HHS and say, well, maybe you want to take a look at this, but here's just some background thinking, I think that's fine.

DR. BRECHER: Other questions or comments?

DR. BIANCO: I just want to support what Karen said. I didn't want to sound negative about what I said. I just wanted to express concern. And this task force has been working in a simply spectacular way. I couldn't say anything less than that. It has been always there, everybody has contributed tremendously, and it includes not only the blood banking--the collection facilities and the government, but also manufacturers. And so it is the entire--as Jeff Miripol was saying this morning, the entire supply chain is there. And that's very, very important.

DR. BRECHER: Okay. If there are no further comments, I think we can move on to discuss the issue of one of our prior recommendations. As many of you recall, in January of this year the Committee made a recommendation that--I'm going to

just hit the highlights, where we recommended that the CMS carrier manual provisions regarding reimbursement for hemophilia clotting factors, that it includes outdated terminology such as "heat-treated variety" and "non-heat-treated variety" clotting factors; and that it was requested that the terminology be updated.

The Committee also wished to reaffirm at that time its previous recommendation regarding recombinant clotting factors and recommended to the Secretary that the Secretary direct CMS to promptly revise the manual provisions regarding reimbursement for hemophilia clotting factors, to remove all insurance barriers to recombinant terminology.

This led to a response from Dr. Carmona, who was the Acting Assistant Secretary at the time, whereby he wrote us in April, wrote the Committee regarding "your recommendation about reimbursement for recombinant clotting factors. I asked NHLBI for an evidence-based review of the effectiveness of recombinant factor versus native or monoclonal

factor. NHLBI reported that its initial review found no studies that showed a definite advantage of one over the other."

Then, the last, he said he would recommend to the Centers for Medicare & Medicaid that they study the cost-effectiveness of recombinant clotting factor to determine if the language contained in its carrier manual should be modified--which is not quite, I think--it was not quite the intent of the original resolution.

This led to some consternation on the part of a variety of players: the National Hemophilia Foundation, ABC, AABB, et cetera. So that Mac McMurtry, myself, Art Lawrence, and Dr. Beato, we all got together in June to discuss this. And this led to a second letter from Dr. Beato, which is included in your package, and maybe we should take a minute to allow people to read that, if they haven't read it already.

[Pause.]

DR. BRECHER: Okay. I'd like to open this topic for discussion by the Committee. Jeanne?

DR. LINDEN: Well, I just have a question. Do we know who wrote this? Because people at this level in my experience never write their own letters, and it looks like the other one was written by Mac.

I mean, if we knew who wrote it, then we could find out better the intent, rather than perhaps overinterpret or misinterpret a few words here.

DR. BRECHER: I think it's fair to say that the content reflects input from a variety of sources to Dr. Beato, who then incorporated this into a letter that she felt comfortable signing.

Is that political?

CAPTAIN McMURTRY: Yes.

DR. BRECHER: Is the Committee satisfied with this response? I think that is the first question. Jeanne?

DR. LINDEN: Well, in follow-up to that, can you give us a little bit more information about the discussions at this meeting and, you know, the language about "while remaining fiscally

responsible," what that is intended to mean?

DR. BRECHER: HHS felt that their mandate is to be fiscally responsible and that they cannot direct CMS to pay for, for example, a product that was out of the ballpark in terms of cost. They have to be fiscally responsible to the citizens of the U.S. And, therefore, they felt that for drugs--and they consider this a drug--they have to look at this in the bigger cost-effectiveness picture, although they are mindful that there are other ramifications of this particular issue, and that is where they--in the second paragraph, the Department talks about their being acutely aware of the concerns of the hemophilia community and they're mindful of the lost generation due to HIV and HCV.

I would be curious what the hemophilia community feels about this letter. Mark, maybe you could comment.

MR. SKINNER: I would be happy to comment, and my comments are not on behalf of NHF or any of the hemophilia organizations. But I did author--I shouldn't necessarily say "author," but I did carry

the original resolution.

The letter that we received back, and certainly read in companion with the letter to Dr. Katz, I think is appropriate for where we are now. There certainly remain issues, and the letters highlight the issues that we've talked about on this and others, that there's a lot of economic issues that spread the gamut. And whether we debate those points in terms of the economics and the priority and what does and doesn't deserve reimbursement, on this point this isn't the place.

I think the important thing about these two letters together is the strong safety statement, which was not in the original response, which was very gratifying to see that affirmed, the commitment to address the outdated language, which was the essence of the original resolution. And, of course, we recognized that that has to go through a process.

And then I think equally importantly is I interpret these that the Committee's original recommendations which gave rise to the

identification of this barrier in the CMS language, which we were seeking removal of, remain intact; that they have not refuted the Committee's original recommendations and said recombinant shouldn't be made available. They've talked about the importance of the therapies. They recognize the economic costs. They recognize the safety.

So I think on balance, when you read it in context with the full debate, it's a reasonable response at this time, but it clearly highlights there's challenges and there are still issues that this Committee needs to address.

DR. BRECHER: Keith?

DR. HOOTS: Just to add to that, I agree with Mark. I think probably the most important sentence is the second one: "There was never any intent to discourage the use of recombinant..."

I think regardless of what happens economically down the road, I think this sentence can be brought back to say, since discouragement can come in any form, including economic discouragement, to remind anyone that HHS' own

record is saying they did not mean to discourage recombinant.

DR. BRECHER: Now we'll take a public comment.

MS. HAMILTON: Jan Hamilton, Hemophilia Federation of America. I agree with what Mark said and the other comments, too.

I do want us to be cautious, though, because it may not have been the intent to discourage payment for recombinant. However, as we all know, the states are in a tremendous economic bind right now, and if that letter had gotten into any of the Medicaid systems, we could have been torpedoed, because we're already fighting that on many state levels right now where they're not wanting to pay for recombinant product.

And I can understand it wasn't the intent, but I think we still need to be careful, very, very careful and very, very much in a monitoring state to see that our feelings about safety are carried through to the proper levels and that there's education on those levels.

When we talk about economics, if you add up the economic impact of what we went through to get recombinant product and the lost generation and the lawsuits and the Ricky Ray money and all of that, it's far more than any difference in paying for recombinant therapy is. And I think that we really have to take that into consideration.

DR. BRECHER: I think this Committee has gone on record multiple times in support of recombinant factor, and I don't think--

MS. HAMILTON: We appreciate that.

DR. BRECHER: And I don't think the Committee has any intent of stepping back from those recommendations. However, we can make those recommendations, and HHS can choose to accept them, ignore them, or countermand them.

In this case, I think on balance this is a letter of support for our recommendations where they do not wish to discourage the use of recombinant factor.

Lola?

DR. LOPES: Just a little question about

how the system works. In the states that are not paying for recombinant, does that mean that a person who wants to use the recombinant gets nothing, or do they get what would have been paid for the plasma-derived product?

DR. BRECHER: Could you answer at the mike? Otherwise, it will not go into the record.

MR. SKINNER: While Jan is walking up, I think the practical effect is that because you're talking about Medicaid patients, they really have no other options. So they're forced to accept what the government would provide for. Whether they just receive the differential and pay the difference out of pocket, I don't know. They probably couldn't and still qualify for Medicaid if they have those kinds of resources.

DR. LOPES: But if someone else could come up with the differential, are we talking about the whole price or just part--

MS. HAMILTON: Well, yes, if somebody else could come up with the differential, they could. But if you think about it, if a patient is on

Medicaid, they can't come up with the difference. You know, just like every patient who's on Medicare they can't really come up with the 20 percent either. You can come up with 20 percent of a lesser product, but not of these products.

DR. LOPES: The reason I'm asking this is because this is a case where it's clear that one product is much superior to the older form of the product. And so the decision is being made that this is not a sort of vanity product or one--

MS. HAMILTON: No.

DR. LOPES: It's being made purely on the fact that it costs a lot. But we do pay for other treatments that cost a lot if they're based on other technologies, like, you know, liver transplants cost a lot, and yet there is no problem about paying for liver transplants.

MS. HAMILTON: And the thing about that, too, is the decision on what kind of product they get then ultimately is not being made by the patient and the physician. It's being made by whoever is paying for it.

DR. LINDEN: It's my understanding with Medicare and Medicaid that they cannot balance bill the patient for additional things that aren't covered, other than established copays. So I think the Medicaid patient basically might in that situation you're describing only have access to the plasma-derived product. I don't think they can say, oh, but I want recombinant because I have this other payer to add additional funds. I don't think that legally can happen.

So I just don't want to leave a misimpression. I believe that's the answer to your question. They would just have limited access to certain types of products.

DR. BRECHER: Chris?

MR. HEALEY: We're lucky to have a representative from CMS here. I wonder if he could elucidate without putting him on the spot too much.

DR. BRECHER: Dr. Bowman? Welcome to the Committee.

DR. BOWMAN: Thank you. I'm not very familiar with the regulatory issues for the

Medicaid balance billing issues, which this question was addressing. I don't believe--I think Jeanne is correct. I don't believe that there's any provision under the standard Medicare program for any type of balance billing.

DR. BRECHER: What about availability in terms of the fraction that is recombinant versus derived from plasma? Will this cause--is there enough plasma-derived factor? So there's an availability--the answer I'm getting is that there's not enough plasma-derived factor currently in the market to meet the needs of hemophilia, and so there comes a question of what can you purchase.

Keith?

DR. HOOTS: There would not be enough plasma-derived Factor VIII in the present market. If everyone who was on recombinant were converted, I mean, that's--there's no question about that. There would be the potential to ramp up production if that were the case. But, again, as I said last time, the implications go far beyond the United States. This is a worldwide market, so it's not

something where you could just say, oh, well, that would be what would happen. It would be one of those expanding entropy problems if that were to--you know, if someone were to try to undertake that experiment.

Furthermore, I think it probably would have then cascading--as Mark talked about earlier this morning, it would have cascading effects in the other direction to things like IVIg and things that drive other plasma collections and stuff.

So I don't even think that is in the realm of availability possible, at least in the present milieu of production. It would require expanding a lot of production facilities that don't presently--that are not presently online, to put it that way.

DR. BRECHER: Larry?

MR. ALLEN: Well, I've been hesitant to bring up this issue about perspective or perception again. This letter does leave the door open, in my perspective, in terms of if there was some reason there was an increase in the cost of, say, recombinant, for whatever the reason. It seems to

me that the door has been left open for there to be some other people excluded from getting the type of drug or treatment that they need.

That concerns me because although they're saying they understand what's happened in the past, this issue of remaining fiscally responsible leaves a big perception in a lot of people's eyes that if one thing--if something does develop and the cost changes, that they're going to be a lot more people that are going to be excluded from buying and using this product. And the mere fact that, as was mentioned earlier, this is not a choice of the patient or the doctor, this is a choice, once again, of the insurance company or the people who are actually paying the bills, then I'm concerned about that.

I'm also concerned about the number of patients who may want to use it but cannot because they're in a state right now that won't pay for it.

DR. BRECHER: I think that it's fair to say that the Committee is concerned about that as well.

Chris?

MR. HEALEY: Just a comment. I know we're talking about some disaster planning today, and there's no doubt it would be a catastrophe if recombinant weren't available and everyone had to switch to plasma-derived therapies. There aren't enough on the market today or facilities available to meet the demand that exists. But I think our efforts might be equally well directed at making sure that doesn't occur, and I think that is a possibility, that we can make sure it doesn't occur through outreach with the payors, through outreach with CMS and through working with our congressional representatives and so forth.

So it's a disaster of a different ilk than bioterrorism or other things like that, and I think it's something that we do have an opportunity to influence.

DR. BRECHER: Yes?

MS. HAGOPIAN: I'm Judy Hagopian, and I speak only for myself at this committee meeting. I'm with HRSA, Health Resources and Services Administration on the Maternal Child Health Bureau,

and I'm the Director of the National Hemophilia program, and we've been hearing from consumers and tracking this issue, and one of the main goals for our federal agency is to ensure equal access to quality care. I think the concern that I see possibly happening is that there is going to be two different standards of care, and that those who do no have sufficient dollars are not going to get access to what MASAC considers to be standard of care.

So what appears to be happening is that there really could be two different levels, or standards of quality of care. So I just wanted to share that since we represent all vulnerable populations.

MR. ALLEN: Any options that you could come up with that--

MS. HAGOPIAN: I don't have any options at this point, but I know that we certainly are very eager to collaborate with CMS and bring together various individuals to let them know how we perceive things and what we--how we perceive the

impact could be. And we're very eager, very much so, and we'd certainly try to be responsive to CMS whenever they seek our opinion.

MR. ALLEN: Just so I understand, you are saying you're hearing from patients who cannot get the product that they and their doctor choose to use?

MS. HAGOPIAN: What we're seeing is that with some of the Medicaid proposed changes, patients will not be able to access recombinant. It's definitely true what this person said over there. I mean, for whatever reason, that is the reality and will be the reality. We don't have--I can't represent HRSA and say we have a position. I'm just telling you what I hear and what I see.

DR. BRECHER: Chris then Mark.

MR. HEALEY: One of the major ways that states limit access is through a vehicle that's called prior authorization, where you have to get prior approval for administration of the therapy. One of the major ways that states could limit access is through this prior authorization process.

I know that a lot of the stakeholders, including industry, including NHF and other organizations, HFA and others, have been very active at the state level, lobbying and advocating against including blood clotting factors and other plasma therapies from these prior authorization processes. So we can get an exemption and a carve-out from these prior authorizations so there won't be any delay in access to the therapy, there won't be any major constraints on access to the therapy.

One success that was recently obtained through a collaborative effort was up in Minnesota where there was a wholesale carve-out of blood clotting factors from the prior authorization requirements. I think there are six or seven states now that carve out clotting factors or blood component products in general. So there's still a lot of work to be done but there has been some progress made. And I know there's a lot of outreach to various stakeholders to try and collaborate to get that done across the board.

DR. BRECHER: Mark.

MR. SKINNER: Shifting back a little bit, kind of what's been a theme of some of my comments and discussions that I've had with some of you, if we went back to look at this kind of as a global issue, because the plasma therapies are really different than the blood, we're looking at a global market where products move and we're looking at global manufacturers. I think understanding the consequences of these impacts globally, the ripple effects back to the U.S., might actually help drive other arguments to the safety of the economic issue in terms of just the capacity to make these decisions. I've suggested a couple times looking at the plasma issue and looking at it in relation to blood and what the global market dynamics are, what the impacts, the benefits, and kind of the constraints that are presented, and how we perhaps ought to really be looking at the plasma market and the plasma therapies in the U.S., and not look at them just simply as a U.S. reimbursement structure, but to understand the economics. It's beyond just the reimbursement. We really need to have a better

understanding of the global dynamics of the market.

If we do that, then that's really part of our role as an education process, and devising a secretary, and that gives us perhaps a stronger basis and another argument that maybe we haven't used to date, as we talked about the availability of both the recombinant therapies, but all the other advances in technology that have come forward in the plasma field.

DR. BRECHER: Other comments, questions?

[No response.]

DR. BRECHER: I think we could move on to the question of HOPPS, and Pierre, I think there will be some resolutions coming from the Committee.

DR. BIANCO: Mark?

DR. BRECHER: Yes, Celso?

DR. BIANCO: What I see is that in the schedule it's for 1:00 p.m. So for instance, Jim McPherson that will speak for ABC is not here. I don't know who Red Cross would be speaking, and I don't know. Karen?

MS. LIPTON: Well, actually, the person we

were going to have is presently speaking at CMS. So we actually have a substitute speaker.

DR. BRECHER: Why don't we go to lunch, an early lunch.

MR. HEALEY: If you did want to make some progress on the topic, we were going to request that there be an opportunity to present some of the plasma issues and how HOPPS impacts them. We do have a brief presentation ready to go, and Julie Birkofer, your favorite star, is ready to do it. So--

DR. BRECHER: All right, Julie. You're up to bat, and then we'll go to lunch. Thank you.

MS. BIRKOFER: Sorry I didn't have this teed up. I was going to do it at the lunch break.

DR. BRECHER: That's okay. We're used to this.

[Laughter.]

MS. BIRKOFER: Maybe we do want to go to lunch while we wait.

[Laughter.]

DR. LINDEN: I just have a sort of

gratuitous suggestion while we're waiting. There's been a lot of problem with identifying who's who. Can we next time maybe have name tags that are more block letters and not so difficult to read?

CAPTAIN McMURTRY: I've gotten that comment also. I think we need to make the tent cards more legible.

DR. BIANCO: It's the aging of the Committee.

[Laughter.]

DR. LOPES: I wanted to make a suggestion too, since we've got a few extra minutes. It seems that we've had, coming from lots of different areas over the last two days, curiosity about--expressions--and we don't know how it works--about how markets, regulation, innovation and safety come together in this area, and I just wonder if this might be a topic for one of our meetings.

DR. BRECHER: Interestingly enough, there is an Agenda Subcommittee, and we did get together after the close of business yesterday, and market issues, market forces was probably the highest item

on our list of possible new topics.

[Pause.]

DR. BRECHER: To begin your talk, maybe give a little background to everybody as to what exactly HOPPS is, since you're to be the first speaker on the topic.

MS. BIRKOFER: Okay, Dr. Brecher. I'd be happy to.

My name is Julie Birkofer. I'm the Director for Health Policy for the Plasma Protein Therapeutic Association, PPTA, and we are very appreciative of the Committee to allow us to share with you our views on this Medicare reimbursement methodology. I am not a reimbursement expert by training. Since being at PPTA October 2001, I have learned a lot, and one of the things I've learned is that the outpatient prospective payment system, PPS, is a system that was put in place by Congress through the budget reconciliation in the mid '90s, late '90s, and it was further improved in BIPA.

The intent of PPS is to be responsive to the Medicare Trust Fund and to assure the viability

of the trust fund into the future. The intent of PPS is to group, bundle, lump therapies, biologics, vaccines, high-tech, drugs into categories. One of the issues that the plasma protein therapeutic industry has been very active on is assuring that our therapies are paid and maintained in separate APC or ambulatory payment classifications. Again, all of our approaches in Medicare and in the state strive to express the unique critical life-saving nature of our therapies, and the fact that they cannot be bundled or clustered or lumped--and the new term of art is "functional equivalents"--that these therapies, because of the unique nature and the critical access points to the user communities, need to have adequate reimbursement.

So HOPPS is a system that is an annually rule-making process that CMS issues a proposed rule, and the intent is to, on an annual basis, ratchet down and control cost. There is pain across the board. There are rarely any winners. One of the first questions I asked my consultant when the proposed rule came out August 6th, is, I

said, "Are there any winners," because that's one of the first things that our stakeholders are interested in, and the answer was no. The oncology category got a little better treatment, but again, drugs, devices, biologicals, high-tech. It also has a system called a pass-through, which is a dedicated pool of money where CMS placed these high-tech drugs and devices, biologics that they weren't quite sure how to price.

When HOPPS first came in in 2000 the rates were based on manufacturer-reported pricing. '01 was the first year that the payment system used actual claims data, and this caused a lot of bumps and reverberations in the market because of some of the inaccuracies and data problems. And as you all who are experts more than me in this area can appreciate, it is very complex and very difficult to do claims-based data collection from hospitals across the United States.

CMS is trying to perfect their data analysis in their claims system, and this year the proposed rule for 2004 that again was just

released, of course when I was on vacation the first week of August, is based on 2002 claims data. So you have a little lag in your data, and you also have some problems in the accuracies of the data.

As you can imagine, hospital claims, billing clerks, it's kind of like, the way I boil it down, garbage in, garbage out. If you have errors in the fundamentals of the billing level within the hospital, for example, these are all infused drugs. If they only bill for the infusion code and not for the drug or the therapy as well, then your claims data will not reflect the cost of the drug, and this is what we are finding, and this is where PPTA has engaged consultants and economists to analyze this claims data, and I think we're about ready for the presentation, so we will be on script in a second.

Basically, the comment period is 60 days. The final rule will likely come out November 1. And we will then launch into a more aggressive strategy. We're now going to go on screen and now you will see repetition.

By overview, PPTA is a standard-setting and advocacy organization. We represent the world's leading manufacturers of plasma protein recombinant analog therapies, and as the Committee discussed earlier, the global nature of our industry as well as the economic viability of the industry. We coordinate strategic outreach to our stakeholder organizations, Congress and the administration.

Again, this is the time frame and it's an annual process. The issues that we are primarily focusing on are rates and classification, classification meaning blood and blood products categories, pass through, new technologies. The impact on our therapies is, you know, people wonder, well, we have small populations. For example the Alpha-One community has 6,000 diagnosed. Well, that's 6,000 total population. 38 percent of that 6,000 are Medicare eligible. Immune Deficiency Foundation, you have approximately 50,000 people that have diagnosis of PIDD, primary immune deficiency disease, 12 percent

of that population. And for hemophilia the numbers are even smaller in Medicare. People wonder why. Well, not to be crass, but the sad fact of the matter is that disabled you cover, but there are not a lot of people over 65. Times have changed the effectiveness of our therapies, but again, you don't have large populations.

People wonder, well, why do you care about HOPPS? Well, we care because Medicare, as you all know, is a model system, and the rates and the strategies and the tactics that they impose on paying for our therapies, private insurers--you'll see Cigna and Aetna modeling their payment structures. And so for that, PPTA has actively worked with our plasma and recombinant users community to assure that the rates assure access. We firmly believe that access directly links to availability of full choice, all products available, and that that links to safety.

As you all discussed earlier, as Chris noted, it would be a disaster, quote, unquote, of a different magnitude, but a large magnitude if the

full range of recombinant and plasma drive therapies were not available. And just to note, the core therapies that we're focusing on are the IVIG, the Alpha-One proteinase inhibitor and the blood clotting factors.

I can't stress enough the unique life-saving nature of the therapies, and you know, people wonder, you know, why are the rates so important, and why as an industry are we concerned that these rates are adequate, and you know, aren't you making enough? Well, what you have to appreciate is that these therapies are manufactured in a very complex and constantly evolving process with constantly increasing regulatory requirements. We are very, very different than our brethren in the pure pharmaceutical industry. These therapies are life-sustaining. They treat chronic and often primarily genetic diseases. There are no alternatives. There are no generics available. These are must-haves. Without these therapies, people, their lives are impacted, and I'll leave those arguments to my colleagues in the consumer

and in our stakeholder organizations. I think they can best deliver those messages.

IVIG, just to give you a quick overview, the permanent APC is maintained, and this is important again because we were not bundled into an infusion category. The issue here is classification. As you recall in the May Advisory Committee, you all expressed a resolution on blood, on IVIG, that the therapy should be put into the blood and blood products category, defined as a blood and blood product. It is, with all due respect to Dr. Bowman, ludicrous to me as a policy person, that CMS does not recognize IVIG as a blood product. HHS has CMS and FDA underneath its umbrella. FDA defines IVIG as a blood product and a biologic. CMS, for some reason, has a different view, and will not recognize IVIG as a blood product. However, it does recognize, blood, blood products, including hemophilia factor. Hemophilia blood clotting factor, as we all know, can be plasma derived or recombinant. The new technology recombinant does not include any proteins.

Therefore, the new technology clotting factors are not necessarily as pure a blood product as IVIG. IVIG is 100 percent solely plasma derived.

From a policy perspective your resolution and report language that was put in by the Labor, HHS Subcommittee on Appropriations, the House Subcommittee, mirrored your recommendation that CMS include IVIG in the classification, blood and blood products. Naturally, part of our advocacy strategy in the next 60 days will be to meet with CMS and to further make an attempt to enlighten them that IVIG is a blood product.

With regard to blood clotting factors, the rates are inadequate to sustain access. We realize, and we understand, that again it's an annual intent to ratchet down costs. For example, plasma-derived Factor VIII had a 10 percent reduction, and recombinant Factor VIII also had a 10 percent reduction. Can the companies sustain this? Perhaps. Again, the economic viability of the industry is at stake. Those of you that look at the plasma protein industry may be aware that

we've had some consolidations, that there's been some layoffs, there's been some reductions. And our industry isn't unique, the entire economy is suffering.

The point is that again these rates have to be adequate to sustain access and it all links availability and safety.

The Alpha One proteinase inhibitor was really, I think, one of the major tragedies of this proposed rule. It was an unexpected event. Again, noting in this rule, nothing in HOPPS, rather, is forever. In this annual rule-making process things change. In 2003 the final rule exempted Alpha One proteinase inhibitor from HOPPS and put it into a cost-based system. This year, because of political pressure, other groups, other disease states, trying to get their therapies exempt from HOPPS, banged on the door and instead of shutting the door to those other groups, CMS in turn rescinded the exemption for the Alpha One and the other three, quote, unquote, true orphans, because again, CMS defines an orphan drug different than the FDA.

It's kind of a little bit more of that disconnect in definitions going on. We firmly believe that the rate that is proposed is inadequate to sustain access. Reasonable cost for this population, the vulnerability, the fragile population, we feel should have been given time to determine the impact on that community.

So in conclusion, we are keeping our eye on October 6, the comment period on the proposed rule. We have already begun the process of working with our stakeholders. We are already in the process of engaging members of Congress to write letters and to work with us in lobbying CMS to hopefully enlighten them on some of these crucial issues to the plasma and recombinant user community. And again, what will give us credibility, as it did last year when we were successful in having CMS recognize the Alpha One, what made us successful is, as an industry, we really stepped up and did some data collection and analysis that focused on the inaccuracies of the claims data. Again, in April to December '02 time

frame, it is not a robust enough data set.

So that is where PPTA, that's our opinions and our take on HOPPS, and November 1, final rule, and hopefully if we're given the opportunity to update you all at your next meeting, we'll have a better story.

I thank you very much.

DR. BRECHER: Thank you, Julie.

Comments or questions from the Committee?

Keith?

DR. HOOTS: Presumably these HOPPS rates are based on the preceding year's outpatient reimbursement rates; is that right? It's not based on hospital rates; is that correct?

MS. BIRKOFER: It's based on hospital claims data from the preceding year. So for this '03 proposed rule, CMS is using April 2002 to December 2002 actual hospital claims data that they take, they aggregate, and then in house they manipulate and apply methodologies and formulas to get the rates.

DR. HOOTS: And why, since they've been

paying on fee-for-service for these products, I mean reimbursement per product, outpatient wise, did they choose to inpatient data to arrive at outpatient reimbursement? Because you've alluded to one of the major issues which is particularly germane, and I provide you actual personal experience, where inpatient billing is far, far less accurate for products of rare disease like hemophilia, because again, you pointed out very astutely, that the people doing that are also billing for cabbage(?) procedures and stuff, and this is not something they know anything about. By contrast the billings that are done say for home care companies are likely to--since that's their literal bread and butter--are much more likely to be accurate and reflective than are inpatient data.

Then of course the other thing is that there are problems in terms of how they're used, because they may be used quite differently in an inpatient than in an outpatient setting, so it seems to me that that's a disconnect that at least for the longer term might need to be suggestively

addressed, particularly since annually it's going to be updated. But if they're going to do it, they ought to do it the right way it seems to me.

Secondly, in terms of--do they specifically, for instance, for IVIG, when they are looking at those claims data, do they limit themselves to a very specific DRG for reimbursement to determine rates, and how accurate do you feel those determinants are? We all know, for instance, that IVIG may be used for everything from ITP to primary immune deficiency, but clearly, if the impact is on reimbursement for immune deficiency, it's in that context that the data should be collected.

MS. BIRKOFER: Thank you, Dr. Hoots. Your point on IVIG, last year when we looked at the data, we did not drill down into the ICD9 code. This year when we look at it we are going to have our consultant pull the entire 279 series. That will hopefully bring to bear data for PIDD only, and that is, our concern and our interest is to assure that the IVIG that is used to treat chronic

disease is accurately and adequately reimbursed.

With respect to the disconnect in the inpatient and outpatient, I appreciate your comment. I totally agree. And Shannon Penberthy and I have spoken on that, and I know Shannon Penberthy, who represents the NHF in town, is going to be very deeply digging into that, and we will work and support her in that effort. So I thank you for that.

DR. BRECHER: Additional comments, questions?

DR. PENNER: Just one quick. There still is only one source of supply for the Alpha One; is that correct; the German company?

MS. BIRKOFER: Actually, sir, just recently within the past month, our member companies, we've had two new entrants into the Alpha One marketplace.

DR. PENNER: So there is a little competition there and maybe resources?

MS. BIRKOFER: It will be starting up, yes. Yes. We're delighted with that.

DR. BRECHER: Okay, thank you, Julie.

Miriam, you want to make your comment now?

MS. O'DAY: Thank you. I'm Miriam O'Day, and I'm here on behalf of the Alpha One community, the Alpha One Foundation, Alpha One Association, and immune deficient IVIG consumers.

Really, Julie gave you the full overview of what happened. In terms of IVIG our biggest concern is that this Advisory Committee did pass a resolution, sent a clear message to CMS. Congress echoed that message by picking up the language in report language to CMS under their appropriations bill, and requested that we be placed in blood and blood products.

The effect of that really is that if there are reductions in cost to the pool, we have a dampening effect. So there's only a certain percentage that they can take us down, and we hope that that would help to ensure access.

I'm really reporting back to the Committee, echoing to you that CMS has not heeded the call that you gave them. We're not asking for

action today, but we want you to know that that's the status of IVIG.

In terms of Alpha One, as you heard from Julie, the proposed ruling is horrible. We find ourselves in the same situation that we were in when the proposed rule came out in '02. We know that these are populations that are widely dispersed, that have low utilization in the hospitals. Again, we're very grateful to be working with the industry on this because they do the technical review, they purchase the claims data, they parse the claims data, and we're looking forward to them drilling down into the ICD9 codes because we think that we'll identify additional flaws in the data.

So for us, again, we're not asking for action from the Advisory Committee today. We're just here to tell you that all of the support that you gave us in the past and the wins that we have, have now been pulled out from underneath us, and we'll be working hard to try to reverse this rule with CMS, and we may be here again in the fall when

we see the final rule, to tell you that we had a terrible outcome, and at that point we'll be asking for action.

But it's a terrible situation for these communities, particularly for the Alpha One community. As you've got more product into the marketplace, you may have less access due to reimbursement. So thank you.

DR. BRECHER: Thank you, Miriam.

If there are no further comments or questions, why don't we adjourn for lunch, and be back at 12:30 or thereabouts.

[Whereupon, at 11:35 a.m., there was a luncheon recess.]

AFTERNOON SESSION

[12:39 p.m.]

DR. BRECHER: Okay. We're going to resume our discussion of HOPPS. Our next presentation is going to be from the American Association of Blood Banks.

MS. WIEGMANN: I'm going to try to modify the version of the statement I made earlier this morning, and I believe that we've passed out a written statement to you, and I will try to summarize since you all have--many of you have seen me before, since this seems to be a recurring problem, the annual payments that CMS is proposing for hospital outpatient services, in particular for blood programs under this HOPPS program.

This year we are particularly troubled that once again the payments that CMS is proposing for blood products are, in fact, being reduced while we know that in reality the cost of blood continues to increase as we have new safety advances introduced. I'm going to use red blood cells, leukoreduced red blood cells, as one

example.

In 2004, CMS is proposing to pay 30 percent less than what leukoreduced blood cells cost in actual figures in 2001. I'm using 2001 figures because that's the last annual data that we have on national-based hospital acquisition costs, and those are drawn from the NBDRC's last biennial survey.

Under that survey, hospitals paid on average $155 for a unit of leukoreduced red blood cells, whereas now CMS in 2004 is proposing to pay $107. Clearly, this is inadequate.

A similar problem is seen in most all of the blood products across the board. For instance, platelets concentrate, CMS is proposing to pay 32 percent less than what we believe the actual cost was in 2001, again, using the figures from NBDRC's biennial survey.

So given these low figures, we at AABB propose that the problem is, again, because CMS is using inadequate hospital claims data, and at your last meeting we heard of the problems that

hospitals have in accurately billing for blood. AABB is trying to address this problem by going out on the road and educating hospitals about how to appropriately bill for blood. We have issued our reimbursement guide, which we hope will help in this effort. And, in addition, we're actively in conversations with CMS about how to improve their guidance documents.

But since those efforts will all take a number of years to reach our goal of improving hospital data, we propose that in the interim CMS go to a system under which they'll pay for blood products based on a reasonable cost basis rather than under the APCs which they're currently using and under which they have to draw from their inadequate existing cost data.

So AABB continues to appreciate the support of this Committee in pointing out the problems that we as a community are facing in the reimbursement arena, and we would urge you to urge the Secretary to have CMS address this problem before the outpatient rule is finalized and goes

into effect in January 2004.

Thank you.

DR. BRECHER: Thank you.

Any comments or questions?

DR. PENNER: Just in view of our similar discussions at the last meeting, I checked with two of my hospitals that were not submitting any information whatsoever on blood costs because it's all bundled and they didn't care and they didn't think there was any need for it. So I don't know how they're getting these figures from areas around the country as to what the actual costs are when many of them are not submitting them.

MS. WIEGMANN: Right, and, in fact, as I think it was maybe pointed out during the last meeting, CMS itself noted in an inpatient rule a couple years ago that in 1997, only 48 percent of hospitals billed for blood using appropriate cost centers. So, obviously, this is a problem, and our answer to that is we're trying to reach out to the hospitals to fix that and make them cognizant of the fact that they need to bill for blood because

all of this is a prospective payment system; and although it may not affect what they're being paid this year, down the road it's going to make a difference on how blood is paid for.

Here in the outpatient setting, which we know is not nearly as significant as the inpatient, but it is something, and it's important that we have as accurate of reimbursement figures as possible; and then also in the inpatient arena, where we know most of our attention is, that the DRGs that use the most blood are accurately recalibrated in the future to take into account the rising costs of blood.

So, yes, we're hearing the same issue, and, you know, it's just an uphill battle in trying to make hospitals see why this matters. And many of them are, you know, turning to us, I think, increasingly to try to get some help on this complicated matter.

DR. BRECHER: Chris?

MR. HEALEY: Teresa, that was really my question. I don't know if this is more appropriate

for you or for Dr. Bowman or whom, but whose responsibility is it to teach people about the proper way to submit claims? I don't know the answer to that?

MS. WIEGMANN: You know, I would argue that it's many of our shared responsibilities. It was funny. I was just at a CMS Advisory Committee this morning, and the Chair of that committee, troublingly to me, to the group said, well, he had some reservations about whether hospitals even read CMS guidance documents.

Well, I would argue that they do, and so it's important on one level to have the agency issue clearer guidance, but at the same time we in the community have responsibilities. We at AABB are trying to reach out to our hospital members to work with them in getting a grasp of the issue. And then blood centers also can play a role in helping to explain to some of their hospital consumers how this all works.

DR. BRECHER: Keith?

DR. HOOTS: Just a comment about

unforeseen consequences of this kind of slippery slope, and you kind of alluded to it in terms of--and so did John, in terms of the bundling, kind of not even unbundling. I think particularly institutions that are focused on providing care to Medicaid and Medicare patients are so used to just kind of getting hit and hit and re-hit and trying to just survive, particularly academic institutions and that sort of thing.

Each time it doesn't look like much compared to the time before, but if you take a subset of individuals like hypertransfusion for sickle cell or something like that, where they're coming in once a week--I mean once every two or three weeks to get hypertransfused, and you've got half a dozen of them, suddenly--you know, a $30 loss to a service doesn't look much, but you multiply it, even for small numbers, and suddenly you get--what happens is the unintended consequence of people who are having to try to make ends meet saying: Why are we taking care of these patients? Why don't we send them across the street?

And even if they do traditionally take care of Medicaid patients, they may say, well, this could go to the county hospital, which then moves it one step down. They keep getting hit.

And it's a real problematic issue that's hitting all of our institutions that are public supported, whether they be universities that are tax-based or medical schools or county hospitals.

But there's this movement--and we were talking a little bit over lunch about this, of cherrypicking, the ones who have the most discretion about which patients they see will clearly say opt out of Medicaid or Medicare, and they'll move it down one step. And there may be then a distribution that's traditionally allowed it to be spread out. But then when the hit starts being amplified, then it goes down to the very lowest common denominator into the one institution or the few institutions that have nowhere else to send the patients. And those institutions then get really problematically hit.

I think that's one of the things we can't

necessarily address in this Committee, but I think we just need to keep all of these things in mind, that it's more than just a $30 increment that gets accounted against an entity. It really does amplify, and the consequences are usually unforeseen down the road.

DR. BRECHER: Lola?

DR. LOPES: I know that with our university hospital, someone is watching literally pennies on things like pencils and notebooks in their purchasing systems. For biologics and other expensive medical equipment, products, is no one watching? Why do we care more about a penny on 100 pencils than about these changes?

DR. HOOTS: Primarily, I think, in all true form--and, again, it's an evolving system, but I think one of the reasons is that institutions are committed by and large by their charter and mission to do certain things. But there comes a time when, if they can't break even, they don't exist.

So up until the point that they hit that essentially point of no return, their mission

demands that they continue to take care of these patients, even if they lose more and more money in the process.

And so it's a very insidious sort of thing. It doesn't happen in a year. It probably doesn't happen in a decade. But if you look at what has happened across the country with institutions like the ones I'm alluding to that are usually tax-supported, they are--this past year large academic institutions have been laying off large amounts of infrastructure first, staff second, and, finally, faculty. And all that does is mean that now they don't have the faculty to take care of some of these patients, even if they were to walk in the door. So then they have a moral justification for saying, well, we can't take care of them, we're going to send them down the street or across town or across the state or across the country.

MS. WIEGMANN: And I would add to that, representing hospital transfusion services, that is the issue that we do hear; that our members are

hearing from their hospitals, you're a cost center, and why do you costs keep going up and up and up? And we try to explain it's because our product is getting safer and safer and that is our duty to our patients, to provide them with the safest possible unit.

But, you know, your fear--I don't think we have any hard data on it yet, but the fear is that ultimately you're going to reduce the staff, you know, and we would argue that then you're going to end up in a circle of problems, you know, potential other blood safety problems of patients getting the wrong unit, et cetera.

DR. LOPES: I'd just say it seems like at our hospital people are tuned to the big new machines that cost more, obviously, but give better results. It's because blood is such a small price thing that we don't see easily, I think, that the reimbursement issue is a very important one for buying a better service. I'm sure they're getting reimbursed for all the new big technologies we have, or better reimbursed.

MS. WIEGMANN: It depends on the technology, probably, but yes.

DR. BRECHER: I think what happens, Lola, is they examine the reimbursement at the time they bring in that new technology, and then over time the reimbursement ratchets down, and then they're stuck losing money on that technology, too.

MS. WIEGMANN: So, in fact, for instance, with blood, this year, you know, they say at CMS they're giving us some special treatment by only reducing our payments by 10 percent. How does that square with reality? It just doesn't. And that's a 10-percent reduction from what they already pay, which is already lower, far lower than we in reality know what a unit of blood costs.

DR. BRECHER: Okay. Paul?

DR. HAAS: This is a little bit tangential, but I think it's come up before, and it's important that even when institutions start trying to pay attention to the so-called cost/benefit, it's not at all unusual for the benefit--the cost to be absorbed, say, in the blood

area, and the benefit is--maybe the patient has gone home. And that doesn't get calculated. That's not in the hospital revenue sheet.

DR. BRECHER: Certainly there is cost shifting and blood is part of the infrastructure, and we're going to have patients that we can operate on now, we'll make money.

Okay. Thank you, Teresa.

Now we'll hear from America's Blood Centers, ABC.

MR. MacPHERSON: You want me?

DR. BRECHER: Yes, you.

MR. MacPHERSON: I'm going to take a slightly different tack and show you something that most of you have never seen before, and hopefully you'll enjoy it. Sometimes something you haven't seen before isn't enjoyable.

I'm going to first echo Teresa's comments only because--or mainly because it's a problem we've been struggling with for the last three years. And, indeed, it wasn't two years ago, I think, that the blood organizations met with CMS

and they admitted that they didn't have the data on which to base their APC rates, DRG increases. And we said the data exists. We said between ABC and Red Cross' data, you have 95 percent of the world in terms of cost to hospitals. Why don't you use that data? And they said, well, we can't use industry data.

So I would urge this Committee, if it does nothing else, to urge the Secretary to take the industry data and independently verify it and then use that data as a more realistic basis, rather than us complaining to CMS that their proposals don't make any sense and CMS says, well, all we're doing is reflecting what the hospitals are telling us in their billing tapes. And as has already been said, the hospitals have no incentive--at least they don't think they have an incentive, certainly in the short term, to correctly code the data because they see this as such a long-term issue, and with the way money is appropriated these days to Medicare and Medicaid, it's done in huge amounts, on a bundled basis, based on what the

hospitals' costs are. And then the hospitals have to figure out how to use it.

But what I want to do is I wanted to show you a few things that, as I said, you probably have not seen before, and hopefully you'll find it interesting because I think it puts this whole issue in perspective.

This is the same slide that Scott Caswell showed, so I'm not going to show it again. I think you all have the handouts for this. This just tells you who we are.

Okay. For the last 20-some years, we have been collecting the fees that our members charge our hospitals and tracking that information on an annual basis, do it all perfectly legally and within antitrust guidelines. And we've never shared this information until about two years ago when MedPAC, sort of the investigative arm or the research arm of CMS, asked us for our data. And so we began to put some trend data together for their purposes, and that's what I want to show you today.

This data is collected yearly. It's

pricing as of September 1st. We get--over 90 percent of the members participate every year, and we actually--the data that I'm going to show you is based upon a uniform database over the last 20 years.

The data is provided--we have provided this data to other organizations before, but as I said, I'm not sure you've ever seen it so I'm going to show it to you.

This is probably the guts of it, and I know it's hard to see. But if you look, it goes from 1984 to 2002. Now, it gets sort of confusing at--oops. There we go. It gets sort of confusing at this end, but let me just explain this line first.

This is the price of vanilla red cells, if you will, over the last 20 years. And what it shows is that the increases were pretty low--and I'll talk about that in a few minutes when I show you another graph. But what we've superimposed on this are major, if you will, safety measures that have been implemented during that period of time.

And despite the implementation of all these tests over the years, what you're seeing is increases, up until recently, in the neighborhood of less than 5 percent per year, which is kind of--which is, frankly, at or below the rate of inflation, which I'll show you in a minute.

Then you see this huge spike over the last couple of years. A lot of this is related to NAT, quite honestly, and a lot of it is related to, if you will excuse the expression, catch-up. Because during this period especially, blood centers, both Red Cross and ABC members, were losing money. And what I don't have a graph of and what I--we don't have that database put together yet, but what we probably can show you at some point in the future is actually the cash reserves or some other markers for blood centers, for our members, over the last 20 years during this same period. And what you would see is that their reserves decreased dramatically during this period of time.

So some of this increase, at least for the ABC members--I can't speak for anybody else, but

we're half the country--is related to, if you will, frankly, catch-up, trying to staunch the losses and to make up for it. But a lot of it is related to HIV NAT and some accumulated expenses that we didn't account for, HCV lookback, et cetera.

This graph is kind of interesting because now we've been tracking leukoreduced blood for the last few years because that, of course, has become more and more of an important product for our members. In fact, it's about 50 to 60 percent at this point. I think it's about 60 percent.

You'll interestingly note the gap between the cost for regular red cells and leukoreduced is narrowing. As it becomes more and more a common product and as the learning curve goes away of trying to ramp up, you'll see that narrowing, and at some point probably the differential will be fairly small if this product--if the vanilla red cells continue to exist.

I won't even explain the green and the yellow lines because it is sort of--it's something new that we're tracking. Just sort of ignore it

because it really is proportionally the same as here. What it does show, though, is that if a blood center supplies 80 percent of a hospital's needs, their price is significantly lower, about 20 percent lower, than the hospital that cherrypicks. And that's just so that the blood centers can plan much better in terms of what the needs are, and the full-service hospital will pay a lower price than the hospital that just does ad hoc shipping. So these graphs are the list pricing.

The next graph shows this data in a different way, and this is probably hard to see the numbers, but, again, they're in front of you. This shows you for the period of 1996 to '97, that the purple line is the consumer price index for health care; the yellow line is the--I'm sorry, the green line, which you can't see, except--I'm sorry. The yellow line is the producer price index for hospitals, and that's another measurement of health care inflation. And then the red line, which you see here--but you don't see here because it's zero--is the average red cell price increase for that

year period, from this period to that period. And so, again, you'll see that it was relatively flat. And if you go back--as I said before, it is very flat. And then in the last few years for the community blood centers, the price--the average increases have been in the neighborhood of over 10 percent. And this is 18.4 percent between 2001 and 2002. And with West Nile virus and some other improvements, that increase is probably going to be only slightly less for this past year when we collect that data.

There are regional differences, of course. The most expensive blood in the country on the average is Hawaii and California, neck and neck with the Southwest area. The cheapest area in the country is in the Northwest, interestingly. That's an artificial number, though, however, because most of the blood centers in the Northwest also manage the blood supply and do the hospital transfusions. And so their costs are bundled in very often with the fact of not only providing the blood to the hospital but actually running the hospital blood

transfusion service. But they are the lowest-cost area, followed by the Southeast.

So the average cost to collect and process--now, let's put that in perspective. Those are the charges. The average cost to collect, process, test, and distribute one unit of whole blood is about $200 right now. That's what the cost is. The majority of that cost is put onto the red blood cell. Additional costs are recovered on the plasma, both for that used for transfusion, which is about half the plasma that's made, and the other half is plasma for further manufacturing. And then random platelets, if they are made, that's another phenomenon that has caused the increase to red cells over the years, is because at one time the average whole blood collection was turned into three products: a platelet, a plasma, and a red cell.

Well, over the years, the majority of platelets now are provided as pheresis platelets, which have their own cost entirely, and so the blood centers don't have random platelets anymore

on which to load costs on. So cost has shifted to the red blood cells, which is another reason for that big shift that you've seen in the last few years, because there have been--most blood centers--the majority of blood centers, not most, have switched entirely to pheresis platelets, or at least the majority of the platelets that they provide.

And cost increases for 2003 will include, as I mentioned already, West Nile virus testing, added recruitment costs to replace donors lost by variant CJD deferrals, and, as Scott Caswell, my associated, mentioned the other day, the impact on that has been far larger than we actually thought, where the donors we're really losing are the repeat good donors. And so we're losing many more donations a year than we're losing donors. And bacterial testing for platelets is going to have a major impact over the next year or two.

The costs could double with pathogen reduction technology. We don't know where that is, frankly, at this point in time. It could be two

years away. It could be five years away or more. It's going to come eventually. But based upon the technologies that are in the works, that could double the price of whole blood collection alone, not talking about anything else. And certainly we'll keep doing testing because these technologies are not foolproof.

Now, double red cell collections could help stabilize costs for a short period of time, and supply certainly, and it's estimated that about 20 percent of collections could be doubled. But that takes a lot of investment, different ways of collecting blood, and the logistics are a bit difficult. You have to have better trained staff than just to do simple phlebotomies. So that will take a while to implement, but that will help give us sort of, if you will, a one-time break in terms of cost rises and perhaps supply problems.

Now, as I mentioned before, this entire database is available to CMS for verification, and we've said that before, and I know Red Cross has the same figures we do. And CMS could take that

and have it independently verified and use that as the basis for their reimbursements.

We're willing to collect any other data that CMS might want. We can get it, at least on the blood center basis. The hospital is a little bit more difficult. On the other hand, we have about 20 members who are also the hospital transfusion services, so at least we could get a sampling of what those costs are.

As I mentioned, we represent about half the blood supply, but we're also in 45 states, so geographically it's a good database, distribution as well. And along with the Red Cross data, as I mentioned, it should be used to help to determine the APC and the DRG market basket updates.

Last, I wanted to thank Jane Starkey, who some of you know, for putting together this data and the slides. She does a remarkable job in lots of things, including writing the newsletter, which you may be familiar with.

Thank you.

DR. BRECHER: Jim, thank you. That was a

very complete data set. In fact, I'd encourage you to publish it somewhere. I think a lot of people would be interested in seeing that.

Questions or comments? Ron?

DR. GILCHER: Jim, your data is as of September 2002.

MR. MacPHERSON: Correct.

DR. GILCHER: And, in fact, new fee increases have been put in place probably by most blood centers as we speak. Do you have an idea of what that average percentage increase has been for this year 2003-2004?

MR. MacPHERSON: For our members, our understanding is that it's in the nature of around 15 percent.

DR. GILCHER: Fifteen?

MR. MacPHERSON: Fifteen. But there's a large disparity because what we saw last year, even though there was an average of an 18- or 19-percent increase, some only raised their fees 3 percent and some raised their fees as much as 30 to 40 percent. It all depends where they were in the cycle

sometimes, but we're going to see a 15-percent increase.

One figure I didn't put on there--and I don't know if it's important--is that the spread between the first quartile and the third quartile, if you know math--I don't, but if you know statistics--is only about $40. So although the range is big, the actual spreads in cost are pretty narrow.

But costs are continuing to increase, and West Nile virus is a very good example. It costs a lot in terms of infrastructure to get ready for that test, and although maybe the test itself only costs about seven or eight bucks to implement per donor, or per donation, if you think about it, that alone is about 4 percent of an increase to the cost of blood--or cost of collection.

DR. BRECHER: Lola?

DR. LOPES: Sorry for what is a really ignorant question. From the public perception, the Red Cross is a major national charity. That means it collects a lot of money other than for products

it sells. I don't think of blood centers like the Mississippi Valley Center in our region as a charity collecting money.

Are there cost differentials between the Red Cross and the other nonprofit centers that come--

MR. MacPHERSON: I don't think I want to answer the question as you've posed it.

DR. LOPES: Okay.

MR. MacPHERSON: But I'll answer it a different way. Red Cross and ABC members have been on cost recovery for blood and not having charitable subsidies for probably since the early 1970s or late 1960s when United Ways were recognizing that the cost of blood was so expensive that they couldn't keep up. I come from Rochester. I started my blood banking career in Rochester, New York, at the Red Cross, and in 1968, 100 percent of the blood supply was covered by United Way fees. By 1975, it was total cost recovery.

MR. MacPHERSON: There really is not much difference in that. Now, Mississippi Valley Blood

Center and all of our members are not-for-profit 501(c)(3) organizations, and a lot of them do fundraising, but not to subsidize the price of blood, but to do buildings, to do equipment acquisitions, to do research, to do special programs. So not to subsidize operations.

DR. BRECHER: Ron?

DR. GILCHER: I want to get back to my point, Jim. I had asked you the question for a reason, and that is really for our Committee members to realize that the number that you saw up here is you need to I think take an average of 10 percent. I just calculated our blood center increase, which went into effect August 1st. It was a 12.9-percent increase on the processing fee for a red cell, which included our single-unit NAT testing for West Nile.

But for the Committee members, I would take that number that you saw, like the 154 on the leukoreduced red cell and add an additional 10 percent on to that, and that will put you into the ballpark, I believe, of where we're going to be for

this coming year.

MR. MacPHERSON: It's a good point, Ron. The other figure I didn't put in, and I'll just mention, in 1990, the cost to collect a unit of whole blood was around $60, and as I mentioned last year, the cost was around $200. So, even if you subtract inflation, you'll see some huge increases, and many of them for or the vast majority of them really are safety related or regulation related, one way or another.

DR. BRECHER: If there are no further comments or questions, thank you.

MR. MacPHERSON: Thank you.

DR. BRECHER: Do we have a representative from the American Red Cross?

MS. SULLIVAN: Hi. My name is Elaine Sullivan, and I am the Director of Reimbursement Policy at the Red Cross. I'm here speaking on behalf of the Red Cross today. Obviously, I don't want to go through all of the information that both Theresa and Jim have already shown you and talked to you about. We would support the comments of

both Theresa and the AABB, as well as some of the comments that Jim has made.

But what I would like to talk to the Committee about is the fact that both--and bring to your attention--both in September of 2002 and again in May of 2003, this Committee has recommended to the Secretary and CMS, that the reimbursement of blood products be based on a methodology that recognizes fair and appropriate payment for blood and blood products, and we would urge the Committee, again, today, during this comment period on the outpatient PPS proposed rule, to once again resurface those recommendations and have them consider an alternative method of payment, rather than the hospital claims data.

Our data also shows data that is similar to Jim's as far as payment. We have shared that data with CMS. CMS has told us their concerns with using industry data, and one of those concerns is that they're concerned about overpaying some hospitals and underpaying some hospitals, based on using average or median data.

One of the reasons why we, to the APC panel this morning, as well as the AABB, have recommended a reasonable cost carveout for blood products out of the APC system is because an individual hospital will be paid their own reasonable cost, and that satisfies one of CMS's concerns with over- and underpaying hospitals based on medians and averages.

So I would encourage this Committee to take action, once again, during this comment period. The comment period closes October 6, but also, at the same time, I just want to make sure that the Committee understands that this is not an unusual request to CMS. CMS, last year, actually paid for orphan drugs through a reasonable cost methodology simply because they did not feel that the data was adequate to reflect the acquisition cost of the product. So it's not that we're asking CMS to do something new that they haven't done before.

So, with that, I will conclude my comments, and I appreciate the Committee's time in

listening to our concerns.

Thanks.

DR. BRECHER: Questions or comments?

[No response.]

DR. BRECHER: I think I'll open the floor to, if there's any public comments on the question of HOPPS, if anyone else wants to make a comment.

MR. SKINNER: Mr. Chairman?

DR. BRECHER: I'm sorry?

MR. SKINNER: Shannon Penberthy, with Mark Associates, wasn't here this morning when you covered the other plasma folks--

DR. BRECHER: Okay.

MR. SKINNER: And she wanted to make some brief comments.

DR. BRECHER: Okay. Shannon?

MS. PENBERTHY: I know we're at the end of the meeting, and so I'll ask the Committee's preference. I do have a PowerPoint that we could quickly put up or we could pass--you'd rather have that?

Sir, we are going to do it.

[Pause.]

MS. PENBERTHY: I very much appreciate this opportunity to talk about the impact of the 2004 HOPPS on clotting factor. I know there were early presentations today when the Committee got a bit ahead of schedule, so I will go through some of the facts and get to the points we wanted to make fairly quickly.

This is very much a way a follow-up from the presentations that we had done in May to further inform the Committee about the ways that these products are reimbursed.

Just a quick recap. There are about 20,000 people in the United States with hemophilia. Five to seven percent are dependent upon Medicare, about 1,100 persons, and we are uniquely recognized in many of the Medicare payment systems. We're the only drug with a passthrough payment status in the hospital inpatient setting, meaning that there's a separate reimbursement for clotting factor that's used in a procedure for a person with hemophilia.

We are one of the few self-administered

drugs covered under Part B, which allows our persons with hemophilia to home infuse, self-infuse at home. And then, under HOPPS, we've been recognized, rather than being bundled with other APCs for drugs or procedures, we do have separate APCs for each of our classification of drugs. It's classified by the HCPCS or J Code.

Hospital outpatient system. The prospective system implemented in August 2000 replaced the previous cost-based system. Last year, was the first year that our products had been truly in the prospective payment part of that. We had been allowed a 3-year period under the passthrough category to allow for collection of data. I'll tell you in a few minutes that didn't really help us.

The proposed rule last year would have leveled reimbursement to 52 cents a unit for virtually all of the products, regardless of whether they were plasma derived or recombinant, which just would have had a devastating effect.

We did respond, providing data that we

collected on hospital cost through our Hemophilia Treatment Center networks. And the final rule did recognize the poor quality of the hospital billing data and implemented a dampening effect to limit reductions. So, for 2003, we were limited to reductions of no more than 15 percent.

The current Year 2004 Rule, proposed earlier this month, it provides more favorable treatment for blood and blood products than other nonpassthrough drugs. Other drugs that are in this category can be reduced based on the reported cost data by as much as 15 percent, plus a quarter of the difference between what the 2003 payment was and what the 2004 payment would have been if they used the reported data. So we will have some drugs in that category, the broad category of nonpassthrough drugs, that could be reduced by as much as 20/30 percent.

Blood and blood products, collectively, were given special recognition, with a 10-percent dampening effect. So we should all be very excited. We were only cut by 10 percent. It could

have been much worse. And as I say, still net decreases.

We're going to see how this chart looks. So these are the rates, 2003 versus 2004. As you can see, pretty much a uniform about 10-percent reduction for all but our you'll see J-7193. That's the Factor IX monoclonal products, which are like the mid-grade product, if you will, for those, and those actually receive a 5-percent increase.

Our concerns. I gave you actually two pieces of paper, and on this one, which was our summary that we provided to the community of the rule, the last page shows actually the data that CMS reports using in aggregate form, and you'll see that the coefficients of variation for our products range from, based on their own analysis, between almost 6,000 and almost 120,000 for your coefficient variations, which means this is really reliable stuff they're using here.

Our volume is low in HOPPS. About six million units were reimbursed between April and December of 2002, which is the time period they're

using, the hospital cost data, and I'll explain that.

Overall, Medicare, according to GAO, in 2001, paid about $105 million for clotting factor used in the home. Similarly, the inpatient volume were much higher than this. People with hemophilia typically don't go to the outpatient services for their care. They're infusing at home before they go in for a procedure, if they've got an outpatient scheduled surgery. They're making arrangements, even though you're not always supposed to do this, to bring factor with them. But, by and large, we're not seeing a lot of people get reimbursed here. Nevertheless, it's concerning, the rates that they want to put in place and the way that they continue to intend to do it.

We have to go back to remembering that HOPPS is intended only to reimburse hospitals for only about 80 percent of their cost in the Outpatient Department. The rule, in compiling the relative values by which they determine the rates, applies an average pharmacy markup. It doesn't

apply, I would say, to blood and to blood products. They've got everything from aspirin with 1,000-percent markup to our products, which we assume hospitals have a relatively low markup for.

Overall, with the rates that I showed you earlier in the presentation, we don't have serious access problems at this point, but certainly if we continued to have 10-percent or 15-percent reductions every year, I would say the rates that are proposed for 2004 are getting pretty close.

We intend to respond to this rule, given that we're now about six weeks out from when that is due. Again, by collecting hospital-cost data through our Hemophilia Treatment Center network, we've asked for data by brand, we've asked for volume by brand or by HCPC Code.

We had, I know a couple of times the question has come up about CMS's refusal or reluctance to take industry data. We participated in the call that they held last week on HOPPS, and they do provide specific instructions about the data they'd like to see from industry-owned

devices, but they only, for us, say they'd like to see more data.

So I asked the question should we presume that for the data they'd like to see for blood and blood products that it look a lot like geographical variation, et cetera; the characteristics of the data they asked for, for devices, and the answer was, yes. So we are going by that in preparing our comments and seeking this data.

We will provide comments. We're going to make those available to our communities, so they can also respond. And we also were seeking collaboration with other organizations. To the extent that clotting factor is included in the category of blood and blood products, we all need to be cognizant of the recommendations that we're making and the impact they could have on the other products, as long as CMS is continuing to treat us all the same.

Finally, future steps. Looking ahead, this is an annual process you're going to have to go through. And we are seeking to partner with

others to improve the accuracy of hospital cost reporting and billing. We feel that that's absolutely crucial, not only in getting the right data for CMS to be able to make decisionmaking, but I think, more importantly, for hospitals to fully recover the costs that they should be recovering for these products, which doesn't always occur and results in the spiraling effect that some of the other Committee members spoke about earlier.

Our proposals in the last few days about trying to collectively come together to better survey hospital-cost data, I was fascinated with the data that ABC has.

And we've actually been reluctant for clotting factor to, at this point, request exemption from the Prospective Payment System, to be reimbursed on a reasonable cost basis because the data is so bad.

I'd be glad to answer any questions that you have.

DR. BRECHER: Karen?

MS. LIPTON: Not really a question, but I

just wanted to let people know that in terms of trying to help hospitals better bill for this, we've been talking with the National Hemophilia Foundation about revising our billing guide out so that it can include some sort of modular information on the plasma products, also.

MS. PENBERTHY: AABB has done a wonderful guide, and so we are looking forward to that partnership. We think it would be very helpful.

DR. BRECHER: Chris?

MR. HEALEY: Shannon, I just wanted to temper maybe your enthusiasm a little bit about your slide, I think it's number six, where you say "provides more favorable treatment for blood and blood products." We heard earlier that IVIG still isn't included in CMS's definition of a blood and blood product--

MS. PENBERTHY: Right.

MR. HEALEY: So we consider that a major oversight, just to remind the Committee of that point.

DR. BRECHER: John?

DR. PENNER: What was the Factor VIIa, at least prescribed on what unit basis, in your table there? The recombinant VIIa.

MS. PENBERTHY: In the table that CMS provided?

DR. PENNER: Yes, just I notice that 1,000--the 83 or something like that, that's per what?

MS. PENBERTHY: Oh, that is per, I'm sorry, that product is packaged in 1.2 micrograms. So that would be per 1.2 micrograms. You're right. The other products are in international units. That product is treated differently.

DR. PENNER: So it's unrelated, but it is allowed to be at least identified separately, based on that.

MS. PENBERTHY: Yes.

DR. PENNER: And then the other issue, I thought there was something about pharmacies, on the blood products that we have, being allowed for Medicare and Medicaid at 3-percent addition to the cost of the product. How does that--

MS. PENBERTHY: For Medicaid, particularly if you're participating in one of the 340(b) programs, if you're a treatment center that is participating in that program, you're allowed only to bill at acquisition, and then whatever the usual customary state-dispensing fee is, which normally is quite nominal because they're thinking of a prescription of pills or aspirin or antibiotics, rather than a very expensive product that requires extensive inventory management costs, special shipping and storage cost. So, for Medicaid, for those products, that dispensing fee, it barely scratches the surface.

We've had some states that have recognized that and put in place different reimbursement mechanisms for the dispensing fee part, if you will. I think Dr. Hoots wants to say something because he's one of those states.

DR. HOOTS: I can tell you that, in Texas, it doesn't matter. We can ship a month's supply of factor, which might be 15,000 units, net cost $12,000 or so, and we'll get reimbursed $200 above

that.

DR. PENNER: I think this holds true, and I didn't know if this was being addressed as a major problem, inasmuch as it really curtails any of the Medicaid programs that we have for the patients who have hemophilia or are requiring some of these blood products in large quantities because it's, from the pharmacy standpoint, they'd just as soon not bother with it.

MS. PENBERTHY: Absolutely. There are, right now, and CMS has another rule that wasn't discussed here that would make revisions to average wholesale price reimbursement, and one of the proposals is that a 5-cent per unit administration fee would be added to the reimbursement rate for clotting factor.

As part of the Medicare prescription drug bill being debated in Congress, both the House and Senate proposals have provisions that would ask for a study of what that administration fee for clotting factor should be. Again, that's only for Medicare, but we could hope that if something was

implemented, it would help us making our case better to the states. There's a lot of work to be done in state Medicaids in this area.

DR. BRECHER: Jeanne?

DR. LINDEN: Could you please explain a little bit more, so I can understand, this table on the third page of your white handout, not the slides, the columns that are headed "minimum cost, maximum cost, mean cost, median cost," could you please explain what those mean. I'm having trouble making sense of them.

MS. PENBERTHY: Sure. This came from the Medicare rule that was issued on HOPPS earlier this month. And it's their reported minimum cost that was reported to them for, that was billed, if you will, by a hospital versus maximum cost, looking at all of the claims.

DR. LINDEN: For a patients' entire stay, everything they used?

MS. PENBERTHY: Well, this is hospital outpatient, so we presume that they came in and had a service and then left. So this would be, yes,

during that stay. And so when they look at the billing, the cost reporting they receive from the hospitals; for example, J7190, the Factor VIII plasma-derived product, the lowest cost that they received a bill for was remarkably 17 cents, and the maximum cost was $2,600, which would have been--probably more reflect a full infusion.

DR. HOOTS: Can I comment? Go ahead.

DR. LINDEN: I mean, I'm still not clear. Somebody used .3 of a unit, and the mean, I mean, this is not per unit? This is total cost? This is still not making sense.

DR. HOOTS: No, it doesn't.

MS. PENBERTHY: This is why I don't want to use the data.

DR. HOOTS: And that's precisely what I think Shannon is trying to say. Clearly, the $2,000 per unit is not true. I mean, no one could get that past anything. That clearly is $2,000 for an infusion. So the coders don't have a clue about this. This is why it's so problematic. The hospital coders, that's what I was trying to say

earlier, to them, a unit may be a box because they don't, again, these are people who are trained to just like at ICD9s and put down a number, and this is not anything like within their spectrum of expertise, and so that's why they get these weird numbers.

MS. PENBERTHY: Medicare guidelines dictate that you bill to the nearest 100 units, but that you bill per unit, and if you infuse 957 units, you bill 957 of that code at this per-unit price.

DR. BRECHER: Shannon, on that same table, look at Factor VIII, this is for 512 days of billing or infusion? What are the days and what are the units column supposed to represent?

MS. PENBERTHY: The days would, because it's an outpatient service, in my mind, that's representing what was reported as the number of claims. We almost could equate that into the number of claims they had. Again, it depends on how they billed, but they're trying to tell us that there were 512 patient days in which a hospital

billed for that code.

DR. BRECHER: That doesn't seem like very many days.

MS. PENBERTHY: There's low volume of this product in the hospital outpatient department. In terms of the units, that was the number of units that, in the aggregate, was billed between April and December 2002 for that code--assuming that someone didn't bill one unit for the $2,600.

DR. HOOTS: Yes, it's really convoluted, and most of this outpatient/inpatient, that's why, I mean, really, hospital outpatient is going to be emergency center, and a lot of that, I mean, the billing there, as you well know, is very problematic to begin with. Some of it may also be outpatient use in day surgeries, although that's not very common, except for the mildest form of hemophilia surgeries, and essentially that's it.

So this doesn't really represent much of anything, in terms of the outpatient use of this product, which is, clearly, as Shannon said, at home.

MS. PENBERTHY: Other questions?

DR. BRECHER: Okay. Thank you, Shannon.

MS. PENBERTHY: Thank you very much for your time.

DR. BRECHER: Members of the Committee, what are we going to do about this? What is the Committee's feeling? In the past, we have made resolutions that have encouraged CMS to use actual costs.

Karen?

MS. LIPTON: I think it's appropriate because it is a comment period, and if we don't weigh in during a comment period, then we might not as well weigh in at all. I don't know that we know exactly how to solve the problem, and we've had two suggestions, and I'm not even sure how different they are, you know, to use the Red Cross and the ABC data or to just base it on reasonable cost, but I think it's something along those lines, and I think it's fairly urgent. I mean, if it weren't so sad, it would be laughable, but it's, you know, it's really, it's really disturbing.

DR. BRECHER: Jerry?

DR. SANDLER: This is my first time as a member of this Committee, and I'm affronted by the way the government is treating this issue. Hospitals are in desperate financial straits. Reasonable proposals are being made by reasonable people, supposedly, that say it's costing us $200. Look, here's the bill for what the transfusions are costing for people that you are supposed to be paying for, and you are not going up, you're going down?

I mean, this is absolutely outrageous, and I think that we should, in very plain language, write to the Secretary and explain to him, in very plain language what a terrible job his department is doing.

DR. BRECHER: Mark?

MR. SKINNER: I hadn't read these before, but I was re-reading the comments that Shannon submitted in her written comments, and in there it states, CMS acknowledges the poor quality of the hospital data for blood and blood products.

And I'm wondering if we could just pick up on the theme and say we agree with them and that, therefore, if they think their data is flawed, we certainly have said that, and if it continues to be flawed, as they acknowledge, then it shouldn't be used. I mean, if that's actually in their written comments that they acknowledge that their data is inadequate, then I don't know how they could justify using it.

DR. BRECHER: Jay?

DR. EPSTEIN: I'm somewhat out of my depth in this subject area and also not a voting member of the Committee, but I'd like to read a candidate statement that I think the Committee could provide, and I think it falls to the Committee management whether it's appropriate or not appropriate for the Committee directly to respond to CMS in the comment period.

So, putting that aside for the moment, the language I would propose is that: Whereas, declining Medicare and Medicaid reimbursement for blood products and plasma derivatives threatens

both the stability of the nation's blood system and individual patient access to medically necessary therapies, the Committee recommends that the Secretary direct CMS to reexamine its framework for cost reimbursement in this product area and provide for passthrough reimbursement as an interim measure based on actual costs.

DR. BIANCO: That's it. We can go home.

[Laughter.]

DR. BRECHER: We have a motion. Do we have a second?

MR. SKINNER: I make the motion.

DR. HOOTS: Second.

DR. BRECHER: Actually, I think the wording is actually quite good, Jay. And I don't think putting it on the screen right now is going to add very much.

DR. PENNER: I think we'd all have to support, from what we've heard today, that there's a need for this sort of proposal to urge a reevaluation.

I think we've dealt with this, though, in

the past on a couple of occasions, and I don't know if it is superimposed onto previous recommendations. We might want to explore that, but at any rate, I think it needs to be addressed again now because we're at a level of urgency that needs the attention.

DR. BRECHER: Keith?

DR. HOOTS: Perhaps we could handle that ex post facto by saying, having a preamble "whereas" statement that says: Whereas, our recommendation such and such, and such and such, on such and such a date, have addressed this issue previously, we wish to reaffirm.

DR. BRECHER: You know, I think that could be covered in the cover letter that I, as Chair, write.

DR. HOOTS: That's good, yes.

DR. BRECHER: Yes, Karen?

MS. LIPTON: I am not the expert in this, and Theresa just said to me, the one thing is that the word "passthrough" has a technical meaning--it doesn't mean anything to me--and so she said base

it on actual cost instead, rather than using a technical term. And I don't, once again, we're getting into this area where--

DR. BRECHER: Can we use the word "passthrough," Dr. Bowman?

DR. BOWMAN: The problem for you, if you use the word "passthrough," as some of you can probably allude to, that passthrough doesn't necessarily guarantee at all that you're going to get reimbursed at the acquisition cost that you're trying to achieve for the providers and the facilities that you're concerned about.

MS. LIPTON: Would you have a recommendation for words, even though you can't vote, and you probably don't even want to participate?

[Laughter.]

DR. BRECHER: But that's off the record, right?

[Laughter.]

DR. BOWMAN: No. We do appreciate the opportunity to participate with your Committee,

actually, and we appreciate that you've extended for us to be here. I can't recommend anything, certainly at this time, because of the comment period with the current outpatient rule, but I would just phrase it in more plain language to just reflect what you really want and not try to use terms like passthrough because there are probably any number of instances where passthrough has not resulted in total reimbursement for the total cost.

DR. BRECHER: I think we have to be crystal clear. I think we have stumbled in the past with our very complicated wording. So whatever we say, let's keep it simple and clear.

MS. LIPTON: Well, then is it the actual acquisition cost of the hospital for these? I mean, I don't--

DR. PENNER: You might want to, the actual costs, and then there has to be at least some handling or management or cost that's added on, which ordinarily is added on in any of the hospital activities. So that has to be at least accounted for in some way.

MS. LIPTON: So it could be an actual cost of acquiring and providing these.

DR. PENNER: Yes. I think that would provide enough so it would be reasonable.

DR. LINDEN: So would you accept that friendly amendment to your nonmotion?

DR. EPSTEIN: Yes, indeed. I actually put passthrough in quotes because I was very uncomfortable.

MR. SKINNER: Mr. Chairman, the one thing that, and maybe I'll see it when I actually read--it's a little small--is I don't recall Jay reading anything that related to the need for enhanced data collection. I mean, the whole aspect of training and the importance of hospitals being helped to understand how to do it, and CMS recognizing the importance of that from their side, I don't know where that role, whether we want to be placing blame or responsibility for that.

DR. BRECHER: Or the use of industry data.

MR. SKINNER: Right. The data piece that I don't think, whether that's mixing two messages

or whether that it's missing from there. I don't recall.

DR. BRECHER: We could recommend, as a second clause, that CMS investigate or consider the use of verifiable industry data in setting their rates. I think that's going to be more reliable than the hospital data for the time being.

MR. SKINNER: Could we add "or support training" or "encourage"; is that a piece that I've heard people say is the importance of people understanding how to bill?

DR. BRECHER: I think that the national organizations are taking care of that. I don't think that we need to take that to the Secretary.

MS. LIPTON: Well, but there is. We have asked for some clarification from CMS, and until we get that guidance, I think we're all still going to be in the dark here.

MS. WIEGMANN: I think that have been in one of your resolutions from last time. If you have the list of resolutions from last time, I think that you included some provision about the

need for guidance at that point in time.

MS. LIPTON: Mark, do you have what we read on that the last time?

I hate to get technical, but when you finally get this, could you put it in like 14-point bold?

[Laughter.]

DR. HOOTS: Can we add didn't you say actual cost for acquisition and dispersal or something like that, provision?

MS. LIPTON: And I said cost of providing to the patient. I don't know what--cost of acquiring and--

DR. HOOTS: Acquiring and providing the product, yes.

[Pause.]

DR. HOOTS: Acquiring and providing the product.

[Pause.]

DR. HOOTS: Acquiring and providing.

DR. BRECHER: I'm sorry, Jerry. Go ahead.

DR. SANDLER: Rather than wordsmithing

this, I'd like to make two suggestions that go into your cover letter. I think this doesn't really show the disparity in this, and I don't want to touch this resolution because I think it's been very well written, but I think something that points out that they're paying hospitals half of what it's costing hospitals should be communicated. As I read this, I don't get the sense of the injustice that is being done, and I think the cover letter should communicate that.

There's no time line. They could blow us off, like they have been for years, and I really think that there should be some time line in the cover letter that communicates that we would really like to be in touch because hospitals are losing an awful lot of money on the people that they're supposed to be paying for every single day.

DR. BRECHER: Mark?

MR. HAAS: Might it make some sense, to follow Jerry's point, to use as an example that third page of Shannon's memo which is the CMS data itself and which shows the disparity?

DR. BRECHER: Well, we can include that in the cover letter. I think I would do that, as well as probably, if we could get a similar letter on blood products, maybe AABB could provide that to us.

MS. LIPTON: We can put together something from our testimony. I know you just had slides, Jim and Elaine, that we didn't see, but I'm sure we could come up with some language that we'd like in there. Does that sound good?

DR. BRECHER: Or a similar table.

MS. LIPTON: I'm sure we could come up with a table.

DR. BRECHER: With the percentage drop and the old price, new price, et cetera.

MR. HEALEY: Mark, if it's appropriate, I'd like to also offer data on IVIG as well and the Alpha One.

DR. BRECHER: Yes.

MR. HEALEY: Just roll in all of those plasma therapies together.

DR. BRECHER: And if everyone could get me

that data in the next couple of days, that would be helpful.

MR. HEALEY: Also, the question I had is either in the cover letter or the language itself will we make reference to the 2004 HOPPS proposed rule? I mean, that's what's really driving this. We might want to make them aware that we're--

DR. BRECHER: Yes, I'll make that clear in the cover letter that that's what we're referring to.

Ron?

DR. GILCHER: As a point of clarification, I discussed this with Jay, changing the word "declining" to, "Whereas, inadequate and further reducing Medicare and Medicaid reimbursement" would clarify declining.

DR. BRECHER: Yes. So we're going to change the word "declining" to "inadequate."

DR. GILCHER: Whereas, further inadequate and further reducing.

DR. BRECHER: Yes, inadequate and further reducing.

[Pause.]

DR. LINDEN: That's not really grammatically correct.

DR. GILCHER: Yes. And further reduction in--

DR. PENNER: I think you better put "inadequate" up front. I think that hits it better. Don't let them dodge that.

DR. LOPES: I suggest "inadequate and still falling reimbursement rates."

DR. BRECHER: We're continually defining it. How about plummeting?

[Laughter.]

DR. BRECHER: It sounds like people will be happy with "further reduction."

Is everybody happy? No? What do you want to--

"Whereas, inadequate and continually decreasing."

[Pause.]

DR. HEATON: Why don't you move it to, "Whereas, further reduction in already inadequate

Medicare/Medicaid--"

DR. BRECHER: Yes, that'll do it.

DR. HEATON: Because that's the key issue. It's going down, and it was never adequate in the first place. "--in already inadequate--" That's perfect. Thank you.

DR. SANDLER: Would we want to get right to the point and say that he directs the Administrator of CMS, so that there's a little more focused accountability? We've got him by his name. He's the Secretary. Now, we're going to CMS in a bit of a blur, and it kind of slips away. We could just say, "Directs the Administrator of CMS." We know who we're talking about, who's got a job to do.

DR. BIANCO: I think it's unnecessary, Jerry. He's the boss. I think the most important part is I think, instead of refining this, is for us to collect the data, as you suggested, so that the inadequacy becomes apparent.

MR. SKINNER: Mark, were we going to add a reference to the data? Because this says what we

want to happen, but it doesn't say at all why.

DR. BRECHER: I think I can cover that in the cover letter that examples of the changing in the reimbursement are illustrated in the accompanying tables. The tables are self-evident.

MR. SKINNER: I guess what I'm saying is this doesn't offer a solution. To the extent that there is a solution, better data, the solution is here, pay the cost, but we don't say why.

DR. BRECHER: Oh, I see.

MR. SKINNER: Maybe it's self-evident.

DR. BIANCO: I would support what Mark jus said, in the sense that there are alternative sources of data at the end.

DR. LOPES: How about you say, "in this product area, and, until better data are available, provide reimbursement based on actual costs"?

MR. HEALEY: I think one thing to think about, though, is, at the end of the day, the claims data, even if they are accurate and correct, may not be the payment mechanism that you want to follow for reimbursement. I mean, there are a host

of different payment mechanisms out there. There's Medicare reform up on the Hill, so there are a lot of different directions this could take by, you know, locking ourselves in or at least proposing that it may lock us in, and I'm just not sure that's where we want to be.

DR. BRECHER: Mark?

MR. SKINNER: I guess what I would say is, and while I think we've identified the data issue, and I think it's the right thing to mention it, if it goes in the cover letter, and if that really is a part of what we want them to address, that cover letter really doesn't become a part of the permanent record. I mean, what we continually publish is just the resolution part.

So, if it's important for posterity that the data issue stay with this recommendation, then I think that it needs to be in here. If we're open to whatever solution they might come back with, then it's fine to leave it out. But if data really is the driver for this, it needs to be there for posterity.

MR. HEALEY: The data is the problem, right? I mean, that's the problem we're facing today.

DR. BRECHER: I think we need to keep our resolutions simple, clean. If we start putting in tables, I think we're going to trip over ourselves.

Jay?

DR. EPSTEIN: I liked an earlier statement along the lines that CMS be directed to consider using validated industry data in making these determinations. I'm not averse also to adding a sentence higher up, recognizing the problem that the current data set available to CMS is recognizably flawed, but I do think that the recommendation per se should focus on the utility of validated industry data.

So I'd like to suggest we add a part.

DR. BRECHER: In that new paragraph underneath that one, "Further, the Committee recommends that CMS utilize validated industrial data regarding costs."

MR. HEALEY: It's really claims data,

right? I mean, that's what you're getting at.

[Chorus of noes.]

MR. HEALEY: Sorry. Sorry. I understand.

DR. BRECHER: Validated industrial cost data.

DR. LOPES: Is the industry then the producer or the producer and the dispenser?

DR. BIANCO: In most cases, the industry is the producer. On occasion, it will be both, but the vast majority of the times it will be the producer.

DR. LOPES: If we focus, though, on the producers, will the hospitals be left out because they're very important in this underreimbursement issue?

DR. BIANCO: I don't think so, Lola, because ultimately the hospital is the one that will now have the opportunity, if this resolution is accepted, to build using the cost data that was generated that let's say the blood centers provided or the manufacturer of IVIG provided.

DR. PENNER: But then they may just end up

with the cost of the product and the industrial cost, and then when the bill is submitted, which includes administration fee and other parts of it, I don't think that's what we want to leave here.

DR. BIANCO: I don't understand the system, but I do understand infusion costs are charged separately, aren't they?

DR. PENNER: No, they're sometimes bundled. You really can't be sure.

MR. HEALEY: And that doesn't work at all for the plasma and therapeutics industry because the manufacturers are not selling directly to the end user. There are a variety of distribution channels. So my suggestion would be validated cost data. Eliminate the word "industrial" and rely on the appropriate sources.

DR. BRECHER: Jay?

DR. EPSTEIN: Perhaps we can bridge that, if it's validated cost data obtainable from product manufacturers and distributors.

DR. BRECHER: Yes, that should do it.

DR. EPSTEIN: Now, that doesn't address

the hospital's administration costs. We may want to--but I think the thing that covers it is manufacturers and distributors.

DR. BRECHER: So validated cost data available from--

DR. EPSTEIN: Product manufacturers and distributors.

DR. BRECHER: Keith?

DR. HOOTS: Yes, I think that's important, and I agree with what Chris said. I think we want to keep this statement discrete from the previous statement.

MS. WIEGMANN: Just as clarification, for the most part with blood components--I can't speak to derivatives--but you're mainly talking, particularly in the outpatient, about just the cost that the hospital pays for the blood product. If a hospital later does some processing to the product, those costs will be incorporated under other lab fees and other processing or the transfusion itself that are separate from the APC payments. Cross-matching, that has separate coding.

DR. BRECHER: Jeanne?

DR. LINDEN: I don't want to slow things down by wordsmithing, but we heard that there was a lot of problems with recombinant products, and I'm wondering whether plasma derivatives would be interpreted as applying to recombinant products. My recollection is the last time we came up with some other term--I don't recall whether it was plasma therapeutics or something that was more all inclusive that didn't imply only human plasma-derived products.

MR. HEALEY: We said plasma therapies or plasma-derived products and their recombinant analogues in the past.

DR. BRECHER: So go up. Say that wording again, Chris.

MR. HEALEY: You can say, "plasma derivatives and their recombinant analogues." We'll see how good your spell check is here.

DR. EPSTEIN: I think we have one superfluous "and" to delete, also. It should say, "blood products, plasma derivatives and recombinant

analogues."

DR. LINDEN: Except that the recombinant analogues only apply to plasma derivatives and not to blood components.

DR. EPSTEIN: Are there any recombinant cellular products? I'm not sure what you're saying, Jeanne.

DR. LINDEN: No, there aren't. That's my point.

DR. EPSTEIN: That's why I put the comma. "Blood products, and plasma derivatives and their recombinant analogues," without a comma.

DR. LINDEN: If you leave the "and," yes, okay. I thought you were deleting it.

MS. LIPTON: You need a comma the first paragraph, third line up, "in the interim" comma.

DR. BRECHER: Let's read this from the top to the bottom, shall we?

"Whereas, further reduction in an already inadequate Medicare/Medicaid reimbursement for blood products, and plasma derivatives and their recombinant analogues--" is that an extra "and" in

there? No. "--threaten both the stability of the nation's blood system and individual patient access to medically necessary therapies, the Committee recommends that the Secretary direct CMS to reexamine its framework for cost reimbursement in this product area, and in the interim provide reimbursement based on actual costs of acquiring and providing the product."

"Further, the Committee recommends that CMS utilize validated cost data available from product manufacturers and distributors.

DR. HOOTS: A comma after "further."

MS. LIPTON: And do you want to say, "Further, the Committee recommends that CMS be directed to utilize," since that's more consistent with the--

DR. BRECHER: Right. So the second clause would be, "Further, the Committee recommends that CMS be directed to utilize validated cost data available from product manufacturers and distributors.

MR. HEALEY: Is it redundant to, at the

very end there, "And distributors in arriving at the actual cost of acquiring and providing the product," directing them for what they need to do to acquire the validated data?

DR. BRECHER: I think it's implicit.

Jay?

DR. EPSTEIN: I think products should be plural at the end of the first paragraph.

DR. BRECHER: Oh, of providing the products.

DR. EPSTEIN: "Providing the products," plural.

DR. BRECHER: Yes, at the end of the first paragraph.

DR. EPSTEIN: Last word in the first paragraph.

DR. BRECHER: All right. All in favor of--well, let's see, does someone want to make a motion to accept it?

Lola?

DR. LOPES: Move.

DR. BRECHER: Second?

DR. BIANCO: Second.

DR. BRECHER: Anyone want to be a third?

[Laughter.]

DR. BRECHER: All in favor?

Thirteen in favor.

All opposed?

Any people not voting? I didn't vote as Chair, but--and the government representatives don't vote. That's right.

Okay. So this is our motion. I will read it in its complete form one last time from the top just to make sure it is in the transcript:

"Whereas, further reduction in already inadequate Medicare/Medicaid reimbursements for blood products, and plasma derivatives and their recombinant analogues threatens both stability of the nation's blood system, and individual patient access to medically necessary therapies, the Committee recommends that the Secretary direct CMS to reexamine its framework for cost reimbursement in this product area and, in the interim, provide reimbursement based on actual cost of acquiring and

providing the products."

"Further, the Committee recommends that CMS be directed to utilize validated cost data available from product manufacturers and distributors."

In the cover letter, I will also include the charts illustrating the price decreases. Is there anything else that--

DR. HOOTS: Yes, our previous recommendations.

DR. BRECHER: Oh, and references to our previous recommendations.

DR. HOOTS: It is kind of a run-on sentence. You might want to--

DR. BRECHER: It is a long sentence--

DR. HOOTS: Cut it into two somewhere, but you can wordsmith that.

MS. LIPTON: And make sure to reference the proposed rule.

DR. BRECHER: Okay. We can do that and, yes, we may split it into two sentences. We'll have to take a look at that.

MS. LIPTON: I didn't mean in the resolution, I meant in the cover letter.

DR. BRECHER: In the cover letter, yes, refer to the HOPPS 2004, yes.

Jay?

DR. EPSTEIN: I just want to come back to Karen's point earlier that the comment period is open now and that we want an action in the comment period. Of course, this recommendation to the Secretary is timely because the Secretary can act at any point, but we do want a communication to go to CMS during the open comment period, and I think it would be appropriate for us to clarify with the Committee management whether the Committee can directly respond to CMS.

CAPTAIN McMURTRY: Dr. Bowman, when does the comment period end?

DR. BOWMAN: October 6.

CAPTAIN McMURTRY: This will go into the Secretary. It will go almost straight from the Secretary's office to CMS for CMS review. For the response that the Secretary will provide to Dr.

Brecher, CMS will have an opportunity to comment on that review. So they will see the comments right away.

DR. BOWMAN: I wouldn't count on a communication like this going from the Secretary down to the appropriate individuals for comments on the OPPS proposed rule. So it would probably be advisable to send it directly to the address that's listed in the Federal Register for the comments.

DR. BRECHER: We can clarify with management--

DR. BOWMAN: Or at least send a copy, I'm sorry.

DR. BRECHER: --whether we can cc this to the Exec Committee.

DR. BOWMAN: Instead of a formal comment, just cc the cover--

DR. BRECHER: Right. We'll clarify whether that would be acceptable to HHS.

Jay?

DR. EPSTEIN: Also, nothing prevents Committee members acting as individuals from

commenting to the docket, and that's another expeditious mechanism.

CAPTAIN McMURTRY: The fact is, in the ethics statement I read yesterday--you all remember that, right?

[Laughter.]

CAPTAIN McMURTRY: You can communicate directly.

DR. BRECHER: Yes, we can.

DR. BOWMAN: You may.

DR. BRECHER: Yes, you may.

MS. LIPTON: As the totally conflicted person on this Committee, I'm just going to--

DR. BRECHER: I want to know who's not conflicted on this Committee.

[Laughter.]

DR. BIANCO: It would be very useful to all of us to have a copy of the final version and cover letter.

DR. BRECHER: When the letter is ready to go, I will e-mail you the text that I will be sending out.

DR. PENNER: Can the Committee just recommend that a copy of this be forwarded, all of those who wish to could vote to agree, and therefore that would be the same as adding a comment during the open comment period? In other words, we're verifying that all of us are--

DR. BRECHER: Yes, I mean, I think it's--

DR. PENNER: You intend to send this on to them.

DR. BRECHER: That would be one way of getting it there, and I think it's legitimate that I cc all of the Committee members the letter that I'm sending to Secretary Thompson. And so what you do with that is at your discretion, but that would be one way of getting it to CMS.

DR. LOPES: But are you saying that you might not necessarily send this to CMS directly, in which case, certainly someone like me, who has no ties to this community or axes to grind, might be deputized to send it as a member of the Committee?

DR. BRECHER: Not deputized.

CAPTAIN McMURTRY: The way I understand

the ethics rules is that you could take a copy of this letter and as a private citizen send it.

DR. LOPES: Okay.

DR. BRECHER: Mark?

MR. SKINNER: I'm wondering, and I don't know if Jay can answer this question, but given that so many of the recommendations in this Committee relate to blood safety and obviously the work of the FDA, I mean, isn't there precedent for that kind of communication from the committee, that either you've carried back formally or informally or have we ever sent anything directly to you from the Committee at the same time it went to the Secretary? Certainly, individuals have carried the same message individually to the FDA. So, I mean, that's a common practice.

DR. EPSTEIN: Well, agencies sometimes share their draft rulemaking with each other and solicit prepublication comments. Additionally, agencies are free to comment back to other agencies in the open comment period. Let me just say that FDA is aware of this proposed rule, and we have

codified some of our thinking on its implications to blood safety.

Now, what you're saying is have we not also at times received communications of that nature directly to the FDA, and the answer is, yes, and certainly we're interested in those comments. To the extent that you think it's FDA business, it's certainly appropriate we comment to FDA.

MR. SKINNER: I wasn't referring to a specific example, just precedent in terms of how the process might work on this issue as it related to CMS.

DR. EPSTEIN: Well, I would say there is no one-to-one link between a comment or a letter you sent to the FDA and what we might or might not do, commenting on another agency's rulemaking, but we're certainly interested in public comments that would bear on blood safety.

DR. BRECHER: We are going to clarify with the administration whether they feel it is appropriate for us to cc this letter to CMS, and if we are told that we can do that, we will officially

do that.

DR. BRECHER: Chris?

MR. HEALEY: The question I have for Jay is would it be appropriate for us to submit comments to FDA, urging you to work with CMS to harmonize definitions of things like orphan drugs and blood and blood products?

DR. EPSTEIN: Sure. I would hope you'd send the same letter to CMS.

When this is cc'd to the members of this Committee, is it appropriate or can we, in some way, get the appropriate address to CMS?

DR. BRECHER: It's in your packet.

Okay. If there are no further questions or comments, we can adjourn early. Okay. We're done.

[Whereupon, at 2:14 p.m., the proceedings were adjourned.]